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Concordance of Patient-Reported Outcomes Measurement Information System (PROMIS) Questionnaires Between Caregivers and Children With DMD

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Objective

To evaluate the appropriateness of using caregivers of patients with Duchenne muscular dystrophy (DMD) as proxies for Patient-Reported Outcomes Measurement Information System (PROMIS) questionnaires

Key Findings

Caregivers can be considered suitable proxies for children when rating PROMIS Mobility and PROMIS Upper Extremity function

BACKGROUND

- The PROMIS scales are generic patient-reported outcomes that quantify the impact of disease on physical, social, or cognitive function[1,2]
- Although self-reporting is considered the gold standard, caregivers frequently report on behalf of patients in many disease states, including DMD[1]
- PROMIS Parent Proxy (PP) questionnaires are being used in DMD studies, with caregivers rating their child's functional ability[3]
- Agreement between caregiver and child on the PROMIS Mobility and Upper Extremity (UE) questionnaires remains largely unknown in DMD[3]

METHODS

Study Population and Data Collection

- PROMIS Mobility (23 items, v1.0; n=41) and UE (29 items, v1.0; n=94) were administered to caregiver–child dyads at Nationwide Children's Hospital (NCH)
- Patient population included male children with DMD age ≥7 years
- Both caregiver and child completed the questionnaires at the same visit, which enabled a simultaneous rating of the patient's physical function

PROMIS Questionnaire

- Each item of the questionnaire is assessed over 5 levels of function or need for assistance:

Levels of function
Able to do the task with no trouble/never
Able to do the task with a little trouble/almost never
Able to do the task with some trouble/sometimes
Able to do the task with a lot of trouble/often
Not able to do the task/almost always

Increasing function

- This analysis focuses on the responses to each item and the total raw scores calculated for each of the PROMIS Questionnaires

Statistical Analysis

- Descriptive analysis for caregiver–child dyad assessments comparing each item of each questionnaire was reported using the following:
  - Percentage of dyads with perfect agreement (same answer)
  - Percentage of dyads with agreement/near agreement (same answer or 1-step difference)
  - Percentage of dyads where the caregiver reported a higher level of functioning than the child (at a 1- to 4-step difference)
  - Percentage of dyads where the caregiver reported a lower level of functioning than the child (at a 1- to 4-step difference)
- Inter-rater reliability between caregivers and children on overall PROMIS raw scores was assessed using intraclass correlation coefficient (ICC) derived in a one-way random effects model **(Scale: to 0.50 = poor; 0.50–0.75 = moderate; 0.75–0.90 = good; 0.90–1.0 = excellent agreement)**
  - Note: a bias is introduced in the ICC analysis on PROMIS UE by the different scoring systems applied to caregiver and children questionnaires (raw scores range from 0–106 and 0–116 for caregiver and child, respectively); this result is therefore indicative only
- Inter-rater reliability analysis between caregiver's and child's responses item by item on both questionnaires was assessed using the Gwet AC1 coefficient to quantify the overall degree of consistency between caregiver and child **(Scale: <0.0 = poor; 0.0–0.2 = slight; 0.2–0.4 = fair; 0.4–0.6 = moderate; 0.6–0.8 = substantial; 0.8–1.0 = almost perfect consistency)**
  - The linear weighting system was used in the calculations to account for the distance between ratings, defined as the number of levels separating the two assessments, therefore penalizing minor disagreements less than large ones
- All analyses were implemented in Stata version 14.2

DISCUSSION

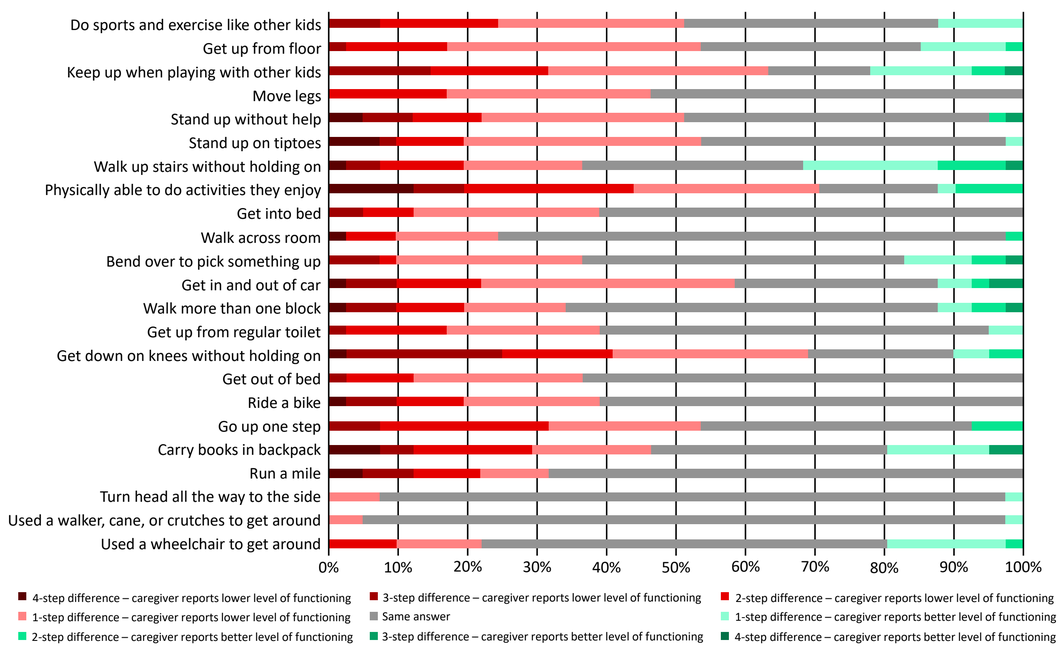
- Assessing consistency in responses item by item for both PROMIS Mobility and UE indicate that most items have substantial to perfect consistency in responses between the caregiver and child
- The items that exhibited largest discrepancy in agreement between caregiver and child (eg, "physically able to do activities they enjoy" or "open rings in school binders") may have less face validity with respect to measuring mobility and UE function (observing enjoyment is abstract; caregiver does not see what the child does at school)
- Items such as opening rings in school binders, tying shoelaces, and dialing a phone appear to be out of date or not applicable to children with DMD
- Consistent with the literature, patient–proxy agreement is generally best for concrete or observable items (eg, walking across room) than less observable ones (eg, being physically able to do activities they enjoy); caregiver-reported function tends to be lower than self-reported function[4]
- The poor-to-moderate ICC observed for the UE overall score is likely impacted by discrepant scoring between the PP and pediatric questionnaire versions; this issue does not apply to PROMIS Mobility assessments
- Strengths of the study include use of Gwet AC1 coefficient with linear weighting and a relatively healthy sample of dyads for rare diseases like DMD
- Moreover, both PROMIS PP Mobility and UE are being refined through Rasch analysis for further relevance in this patient population

RESULTS

PROMIS Mobility in DMD

Analysis of differences between caregiver and child assessment

Shades of red indicate the caregiver rated the child lower and shades of green indicate the caregiver rated the child higher than the child rated himself. The large gray area indicates agreement between the 2 groups



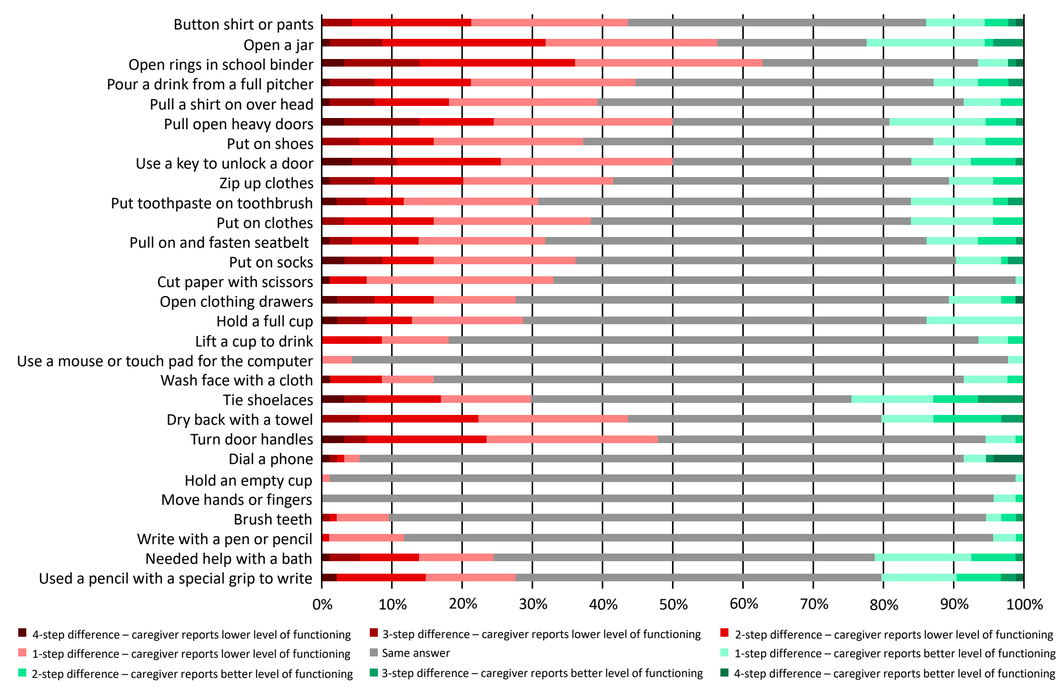
Data for simultaneous PROMIS Mobility assessments were available on 41 dyads

- Children with DMD were 7–17 years old with a mean age of 11.5 years
- Answers to "Used a walker, cane, or crutches to get around" and "Turn head all the way to the side" were in agreement/near agreement between dyads
- The item for which agreement or near agreement is observed the least is being "Physically able to do activities they enjoy"
  - Expectations of caregivers on this subjective item may differ from those of the children
- The ICC for overall PROMIS Mobility scores showed moderate inter-rater reliability (0.555; 95% CI; 0.304–0.735)

PROMIS Upper Extremity in DMD

Analysis of differences between caregiver and child assessment

Shades of red indicate the caregiver rated the child lower and shades of green indicate the caregiver rated the child higher than the child rated himself. The large gray area indicates agreement between the 2 groups



Data for simultaneous PROMIS UE assessments were available on 94 dyads

- At the time of PROMIS UE assessment, children were 7–22 years old, with a mean age of 12.7 years
- Answers to "Use a mouse or touchpad for the computer" and "Hold an empty cup" were in agreement/near agreement between dyads
- The items for which agreement or near agreement is observed the least often were "Open rings in a school binder," "Open a jar," and "Dry back with a towel"
- ICC scores for overall PROMIS UE scores showed poor to moderate inter-rater reliability between caregiver and child (0.413; 95% CI, 0.231–0.567)

Degree of Consistency Between Caregiver and Child for PROMIS Mobility and UE Items Using Gwet AC1

Level of consistency	PROMIS Mobility Inter-rater reliability with linear weighting n (%)	PROMIS UE Inter-rater reliability with linear weighting n (%)
Poor consistency	–	–
Slight consistency	3 (13%)	–
Fair consistency	2 (9%)	5 (17%)
Moderate consistency	8 (35%)	7 (24%)
Substantial consistency	6 (26%)	8 (28%)
Almost perfect consistency	4 (17%)	9 (31%)

- Caregiver and child ratings for **PROMIS Mobility** demonstrated **substantial to almost perfect consistency for 10/23 (43%)** and **moderate consistency for 8/23 (35%)** Mobility items
- For **PROMIS UE**, **17/29 items (59%) demonstrated substantial to almost perfect consistency and 7/29 (24%) demonstrated moderate consistency**

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