

# Rasch Analysis of the PROMIS Parent Proxy Mobility Item Bank Administered to Caregivers of Patients With Duchenne Muscular Dystrophy

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## BACKGROUND

- Patient-reported outcomes (PROs) are recognized by regulators, clinicians, and payers as valuable tools to ascertain meaningful impact of a disease and treatments from the patient or caregiver perspective
- A number of generic and condition-specific PRO measures have been used in studies of Duchenne muscular dystrophy (DMD); however, concerns have been raised about their validity and reliability to comprehensively assess health-related quality of life (HRQoL) in patients with DMD<sup>1</sup>
- PROMIS questionnaires are generic PROs created to quantify the impact of disease on a multitude of HRQoL domains, such as physical, social, cognitive, or emotional function<sup>2</sup>
- Although included in the National Institutes of Health toolbox of recommended measures, one cannot assume the PROMIS tools are valid and reliable for all clinical contexts
- To date, no study has evaluated the content validity of PROMIS measures in assessing HRQoL in patients with DMD
- This study explores the validity and psychometric performance of the PROMIS Parent Proxy Physical Functioning – **Mobility** scale in a cohort of patients with DMD through Rasch statistical analysis

### What Is Rasch Analysis?

- Rasch Measurement Theory<sup>3</sup> is a family of statistical models used to assess the internal functioning of instruments and items
- In the Rasch models, the probability of a specified response is modeled as a function of person (respondent) and item (question) parameters
- The Rasch models can also provide information about how well items within the scale work to measure the ability or latent trait
- The models yield statistics describing the reliability and validity of conclusions derived from items and total scores and thus offer insights to refine and improve instruments

## METHOD DETAILS

### Study Population and Data Collection

- The caregivers of 99 male ambulatory patients aged between 4 to 12 years who had been clinically diagnosed with DMD were surveyed during routine clinic visits at Nationwide Children's Hospital
- Given the young age of the patients with DMD, caregivers served as proxies for patient reports
- A total of 151 observations were collected for this analysis, equally split between patients aged 4 to 7 and 8 to 12 years

### Study Questionnaire

- Generic PROMIS Parent Proxy Mobility Item Bank (version 1.0; 23 items)
- Parent proxies reported on the ease of doing a functional activity via a 5-level Likert scale to indicate how much trouble their child had with each activity (0=unable, 1=with a lot of trouble, 2=with some trouble, 3=with a little trouble, 4=with no trouble)

### Statistical Analyses

- Psychometric analysis was performed using RUMM 2030 software

## RESULTS

### Preliminary Analysis

- A preliminary Rasch analysis of all 23 items showed that only 12/23 items have ordered (and distinct) thresholds (boundaries between response categories) and 11 have disordered (when a person with greater ability does not consistently choose a higher response options [i.e., 3 or 4] for any given item) thresholds

A significant chi-square test ( $P < 0.001$ ) indicated that the data did not fit the models and items were not working as expected across different levels of mobility in the patient sample

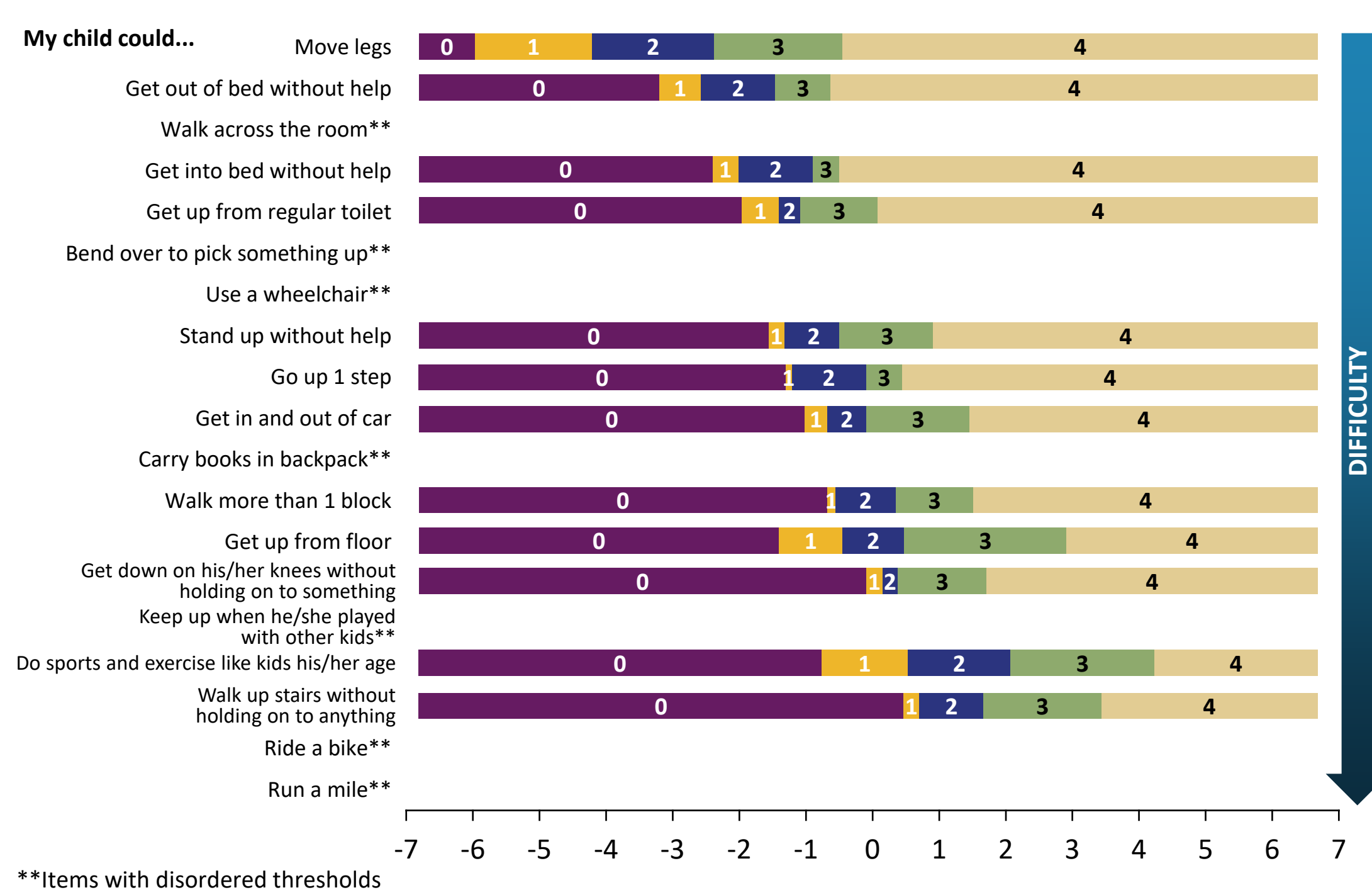
To resolve the misfit, the following criteria were explored:

- Clinical relevance of items
- Ordering of thresholds (response options)

Three expert neuromuscular physical therapists examined the preliminary results, which resulted in the removal of 4 items deemed clinically irrelevant in the DMD population

- Ability to stand on tiptoes; physical ability to do the activities they enjoy the most; ability to turn their head all the way to the side; ability to use a walker, cane, or crutches to get around

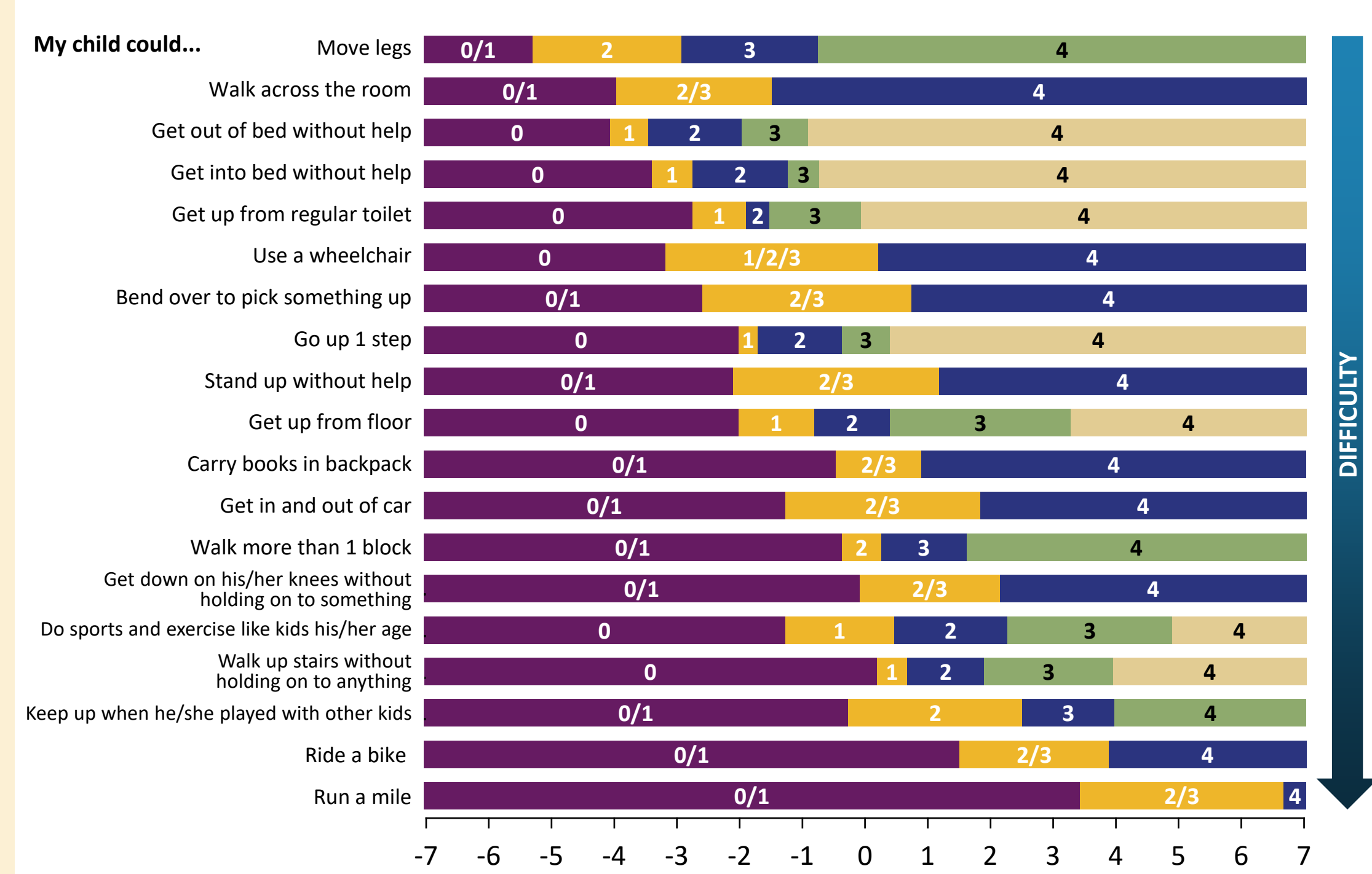
The fit to the expectations of the Rasch models was not reached after the removal of 4 items; the threshold map for the remaining 19 items is shown below



Items showing disordered categories were rescored based on items' probability curves and clinical opinion:

- Three items were grouped in 4 categories, 9 items were grouped in 3 categories, and 7 items (the clinically most poignant items) were left granular with the original 5 response categories

The threshold map for the rescored questionnaire was plotted showing items ordered by difficulty

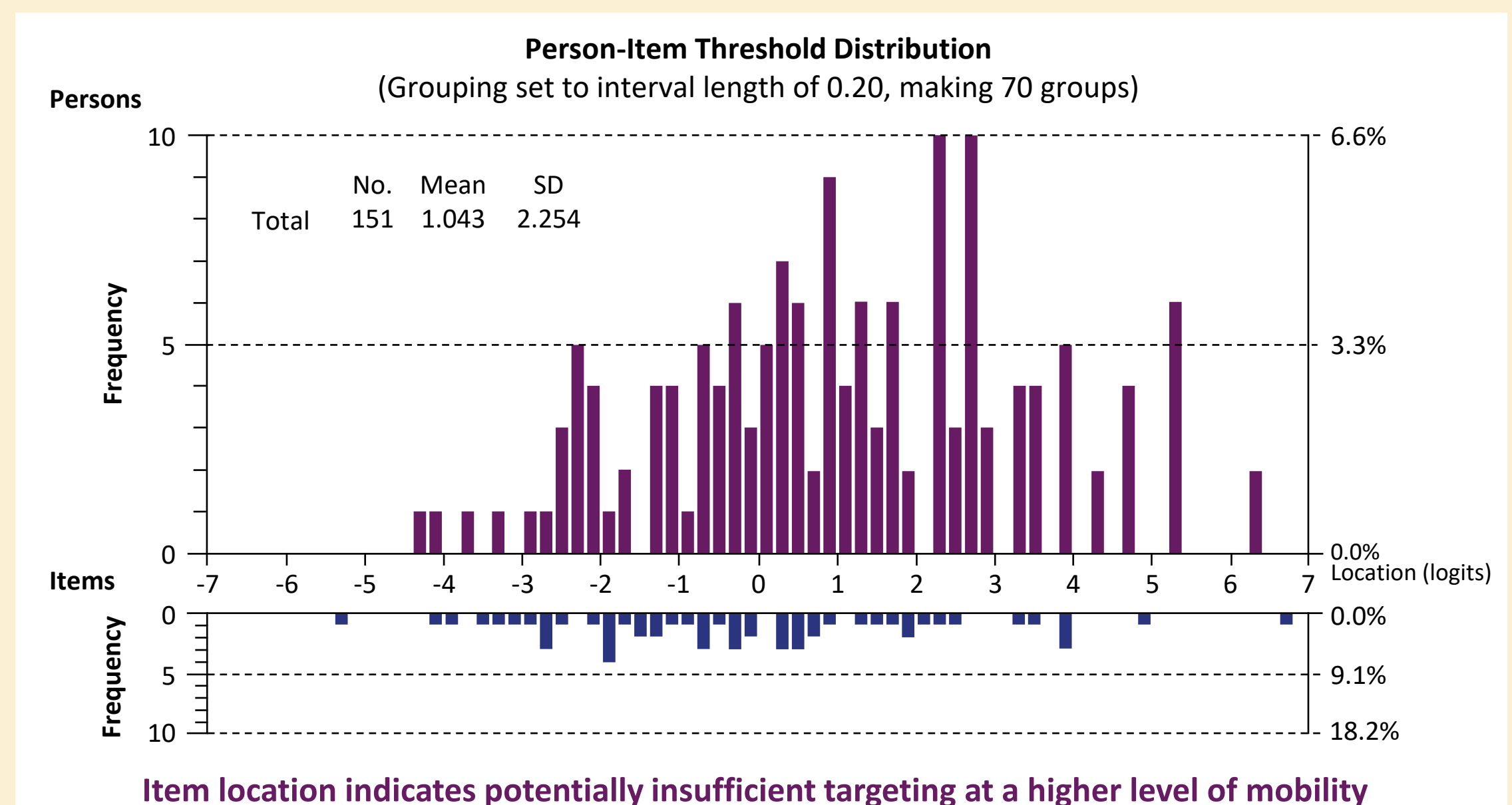


The summary interaction statistics after recoding reflect good fit of the observed data to the model's expectations and the power of the scale to discriminate among respondents with different levels of mobility

- Item trait separation analysis: non-significant  $P=0.143$
- Reliability index (person separation index): 0.95
- No indication of misfit for any of the items (all residual values more than 2.5 or less than -2.5)

The person-item threshold distribution of the rescored PROMIS Mobility is shown below, where person ability (level of mobility) and item threshold (difficulty) are plotted on the same interval logit scale

- Patients with the highest level of mobility and the most difficult items are plotted on the right side of the histogram, whereas those with the lowest level of mobility and the least difficult items are plotted on the left side



## CONCLUSIONS

- The original 23-item PROMIS Mobility scale did not sufficiently fit the Rasch model expectations
- Clinical judgement suggested the removal of 4 items to ensure clinical and practical relevance of the scale
- As suggested by the items' disordered categories probability curves and clinical consensus, collapsing items from the original 5 levels to either 4 or 3 levels restored response across items
- The resulting Rasch PROMIS Mobility scale with 19 items demonstrated a good fit to the Rasch models
- Mobility was well targeted across the severity spectrum except at the higher level; however, lack of sensitivity to detecting higher function is likely less significant when the scale is used on clinical populations such as patients with DMD with mobility limitations. Additional higher-level items are being piloted in the clinical setting
- Limitations of the study include that some patients may not have had a genetically confirmed diagnosis, the population sampled mostly experienced the mild-to-moderate range of symptom burden, and the study focused on proxy reports as opposed to self-reports
- The PROMIS Mobility scale is clinically and patient relevant and may serve as a reliable option for assessment of mobility in DMD from the patient perspective

### REFERENCES

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3. Boone WJ. *CBE Life Sci Educ*. 2016 Winter;15:rm4.

### ACKNOWLEDGMENTS & DISCLOSURES

**Disclosures:** LL, NR, LA, MI: Employees of the Nationwide Children's Hospital and have provided the data for this study; Nationwide Children's Hospital receives grant funding for other research initiatives from Sarepta Therapeutics, Inc. CLR: Independent biostatistician who received funding from Sarepta Therapeutics, Inc. to help with the analysis. IA, KG: Employees of Sarepta Therapeutics, Inc.