

Validity and Reliability of the EQ-5D-5L and PROPr in Patients with Spinal Muscular Atrophy

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1. INTRODUCTION

Although preference-based measures show satisfactory validity to support economic evaluation in several populations and patient groups, extant literature does not provide sufficient evidence that supports the EuroQol five-dimension questionnaire (EQ-5D) or the Patient-Reported Outcomes Measure Information System Preference measure (PROPr) among individuals with rare diseases, such as spinal muscular atrophy (SMA), one of the most prevalent monogenic neuromuscular disorders caused by loss-of-function mutations. ‡

2.OBJECTIVES

- To assess the psychometric properties of the five response-level of EQ-5D and the 29-item version of the Patient-Reported Outcomes Measure Information System (PROMIS-29) in patients with SMA.
- To compare measurement properties of the two measures in this patient group. ‡

3. METHODS

»Data Source

A web-based survey coordinated by a national SMA patient organization in 2021.

»Participants:

Individuals who were formally diagnosed with SMA, aged ≥ 16 year-olds, having no cognitive problems, and able to provide informed consent.

»Assessment:

- Ceiling and Floor effects.
- Convergent and divergent validity.
- Known-group validity of the EQ-5D-5L and PROMIS-29.
- Agreement between EQ-5D and PROPr.

4. RESULTS

A total of 137 individuals (Age: 16 ~ 47 years; female: 52.6%) with SMA completed the questionnaires. No participant reported the best (11111) or worst health state of the EQ-5D-5L.

Table 1. Profile of EQ-5D and PROPr

Variable	Ceiling effect	Floor effect	Mean	Range
EQ-5D utility score	0.7%	0.7%	0.27	-0.35-0.75
PROPr	0.7%	0.7%	0.25	0.04-0.93

Fig 1. Distribution of EQ-5D utility score

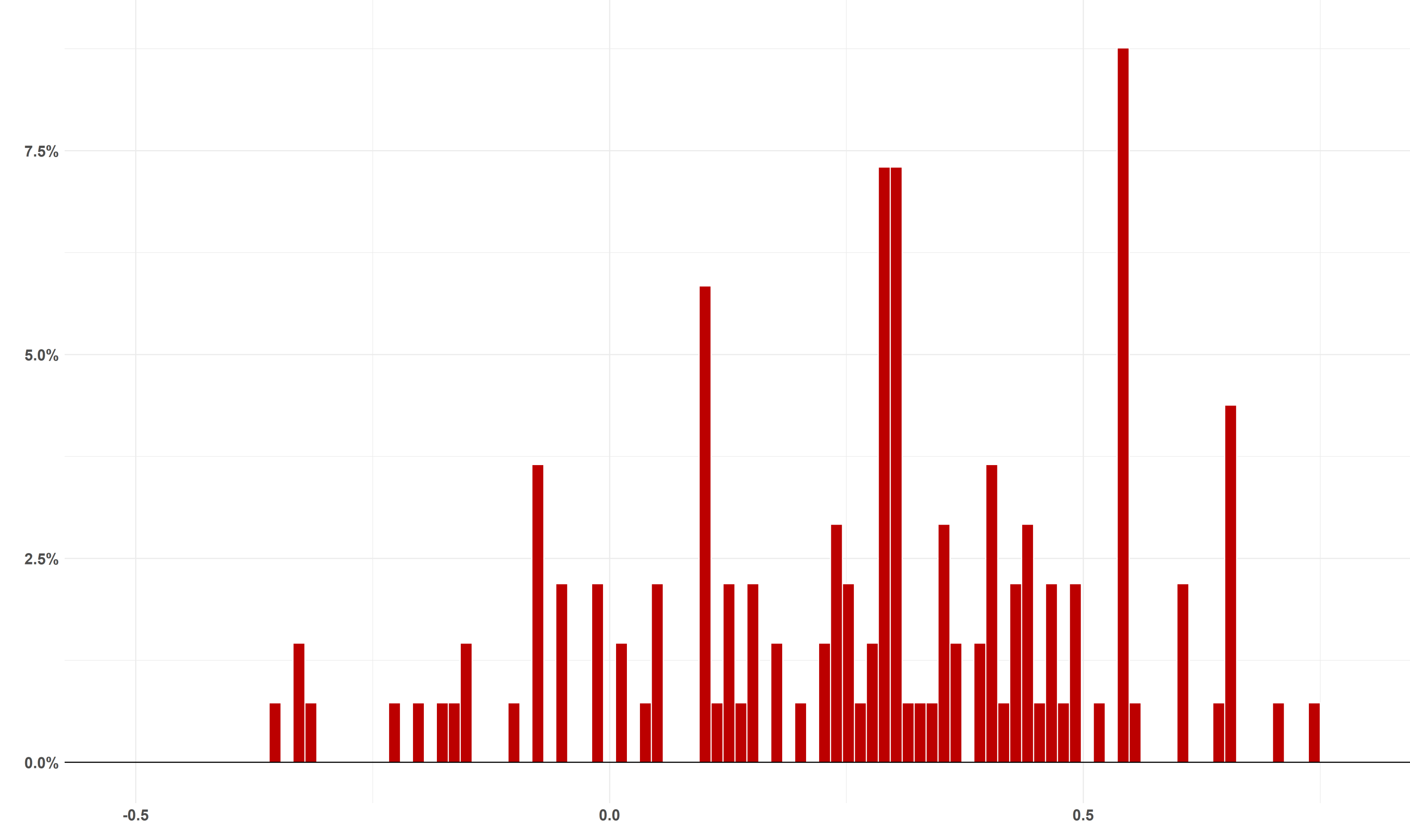


Fig 2. Distribution of PROPr

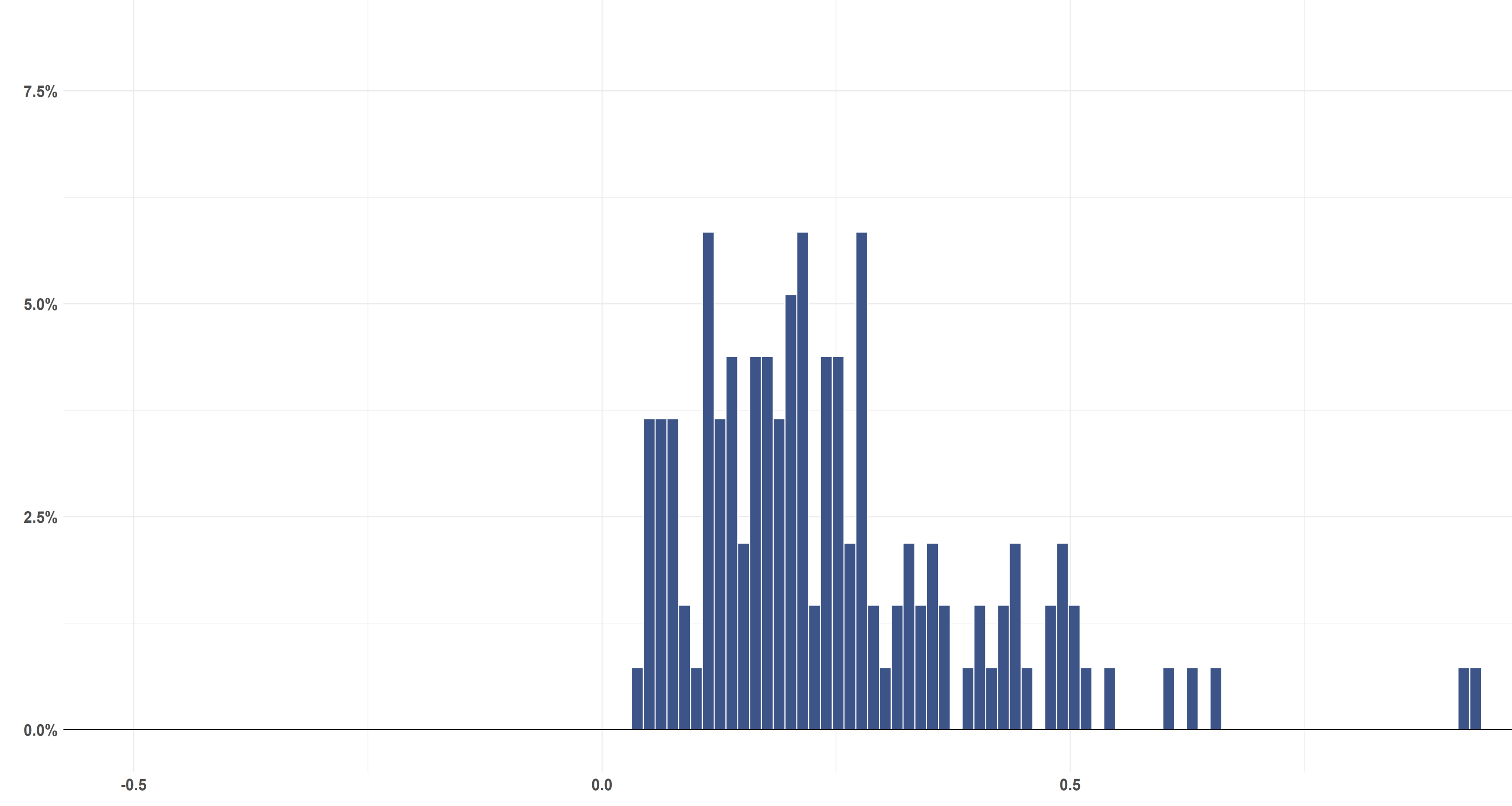


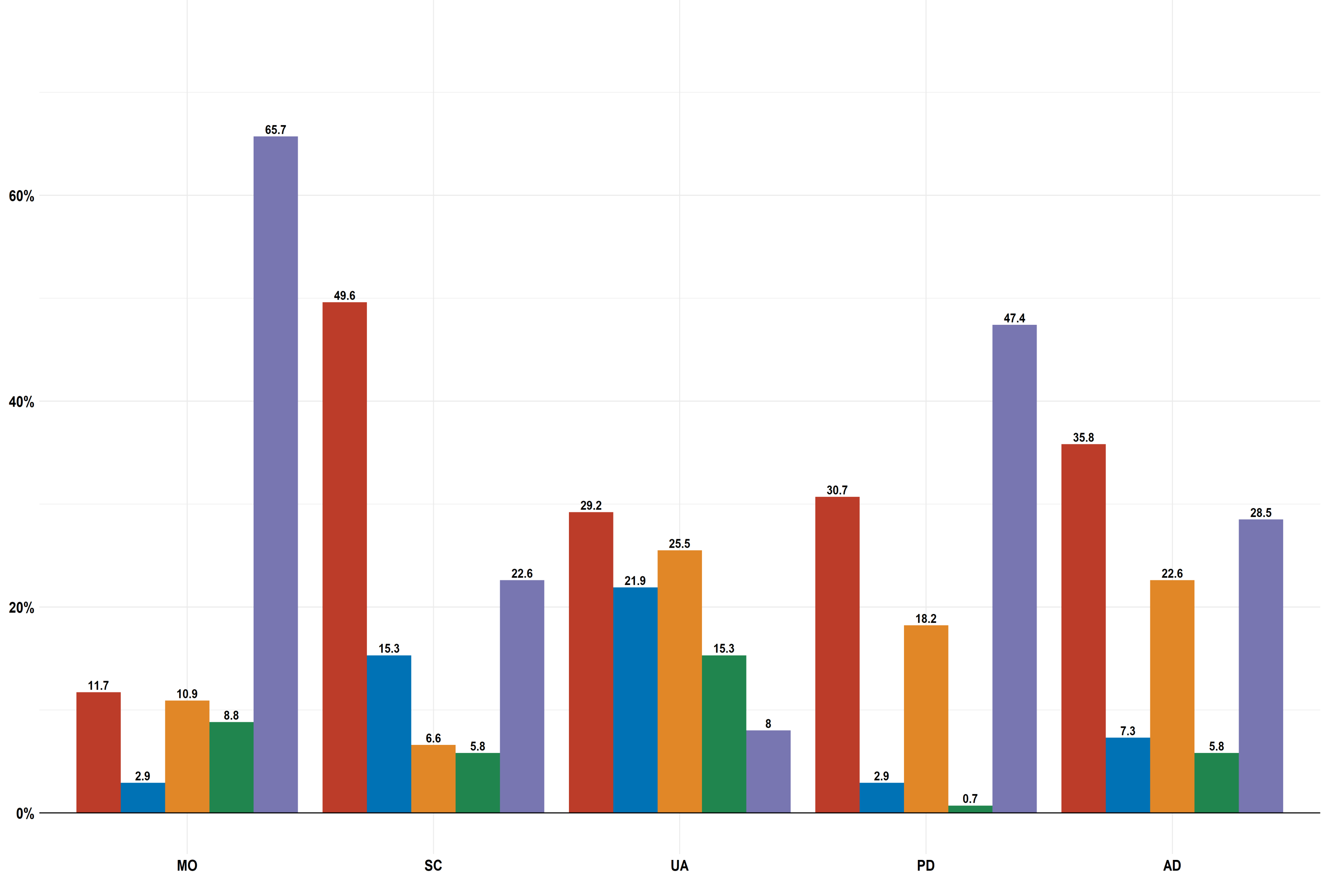
Table 2. Convergent validity between EQ-5D-5L and PROMIS

EQ-5D Utility score, PROPr and SMAIS	Correlation coefficient (95% C.I.)
EQ-5D Utility score vs. SMAIS	0.28 (0.11, 0.41) ***
PROPr vs. SMAIS	0.38 (0.2, 0.49) ***
Agreement between EQ-5D utility score and PROPr (ICC)	
EQ-5D Utility score vs. PROPr	0.4 (0.31, 0.57)

*** $p < 0.001$

All hypothesized were confirmed, and dimensions of the EQ-5D-5L were significantly correlated with corresponding subscales of the PROMIS-29, supporting convergent validity. ‡

Fig 3. Responses for dimensions of EQ-5D



5. CONCLUSION

The EQ-5D-5L and PROPr assessed by the PROMIS-29 are valid preference-based measures for individuals with SMA. The PROPr yields a considerably lower utility score than the EQ-5D-5L in this patient group. Their performance should be further explored in the other rare disease groups. ‡

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