Interim Validity and Reliability Testing of the Pediatric Narcolepsy Patient-Reported Outcomes Scale

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Introduction

- Pediatric narcolepsy patient-reported outcome measures needed to accurately assess problematic symptoms and disease burden, determine treatment efficacy, and identify unmet needs to guide future therapies
- We developed a pediatric narcolepsy patient reported outcome measure (PN-PROS) through focus groups, literature review, and expert opinion, with 55 questions (27 symptom and 28 function) identified for field testing
- This study evaluated validity and reliability of PN-PROS for patients 9-17 years of age in a North American English-speaking population

Methods

Participants:
- Five North American sites: Boston Children’s Hospital, Toronto Sick Kids, Geisinger Health, Stanford Medical Center, patient advocacy websites
- Patients with Narcolepsy Type 1 (NT1), Narcolepsy Type 2 (NT2), and Obstructive Sleep Apnea (OSA)

Protocol:
- Electronic consent and surveys completed through REDCap
- Participants received $50 gift card for completion
- Participants re-tested on PN-PROS 1 week later

Psychometric Analysis:
- Classic Test Theory Validation
- Internal consistency based on factor analysis, convergent/discriminative validity, criterion validity using ESS-CHAD, Peds QL, Peds PROMIS Life Satisfaction
- Test-retest reliability

Results

- Symptoms Domains: Behavior, Attention, Wake, Sleep
  - Cataplexy domain removed to achieve fit
- Function Domains: Problems, School Function, Social Function, General Function
  - Cataplexy domain removed to achieve fit
- General Reliability: Symptom Scale (0.85) and Function Scale (0.91)
- Fit Statistics Symptom Scale: RMSEA 0.05, SRMSR 0.09, CFI 0.96
- Fit Statistics Function Scale: RMSEA 0.06, SRMSR 0.11, CFI 0.92
- Known group validity (Fig. 1) and criterion validity (Fig. 2 and 3) were very good, but criterion validity with MSLT was poor (r=-0.12, p=0.47),
- Test-retest reliability (n=173, r=0.90, p<0.001) excellent

Conclusions

- PN-PROS interim data show good validity and reliability
- Goodness of fit depends on removing cataplexy questions, suggesting high variability in perceived daily impact
- We anticipate making separate questionnaire for cataplexy symptoms and function
- Limitation: Most participants treated with medications
- Need assistance with recruitment of more patients!

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