Title: Interim Validity and Reliability of The Pediatric Narcolepsy Patient-Reported Outcomes Scale (PN-PROS)

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Background: There are currently no validated, patient-reported outcome measures for pediatric narcolepsy that assess disease burden, determine treatment efficacy, and guide future drug development. To ensure optimal clinical management of pediatric narcolepsy, we developed the Pediatric Narcolepsy Patient-Reported Outcomes Scale (PN-PROS) through literature review, content expert interviews, patient focus groups, and cognitive testing with patients (ages 9-17 years) and their parents. The resulting PN-PROS item bank included 55 items that assess pediatric narcolepsy symptoms and their functional impact. The aim of this study is to provide interim data from our multi-site validation and reliability study of the PN-PROS.

Methods: We performed field testing for validity and reliability of the PN-PROs in pediatric narcolepsy patients (9-17 years) with a comparator group of pediatric obstructive sleep apnea patients. We recruited participants from Boston Children’s Hospital, Stanford Medical Center, Toronto Hospital for Sick Children (SickKids), and Geisinger Medical Center, as well as from narcolepsy patient advocacy meetings and websites. Participants completed the PN-PROS, Epworth Sleepiness Scale for Children and Adolescents (ESS-CHAD), Peds QL, and PROMIS Life Satisfaction using the REDCap data capture platform. Participants completed the PN-PROS item bank 1 week later for test-retest reliability.

Results: To date, 83 pediatric patients with narcolepsy (mean age=15(2) years, 52.3% female, 26% non-Caucasian) and 60 pediatric patients with OSA (mean age=13.2(2.6), 46.3% female, 22% non-Caucasian) have completed all study measures. Discriminant Validity: Participants with narcolepsy reported a higher PN-PROS mean total score than participants with OSA [narcolepsy=126.9 (28.6), OSA=95 (31.2), p<0.001]; results retain significance controlling for age, race and gender [group main effect: F=36.6, p<0.001]. Content Validity: For participants with narcolepsy, the PN-PROS total score was significantly correlated with the ESS-CHAD (r=0.64, p<0.001), Peds QL (r=-0.84, p<0.001), and PROMIS Life Satisfaction (r=-0.55, p<0.001). For participants with OSA, the PN-PROS total score was significantly correlated with the ESS-CHAD (r=0.65, p<0.001), Peds QL (r=-0.81, p<0.001), and PROMIS Life Satisfaction (r=-0.36, p<0.001). Reliability: Internal consistent was strong for both participants with narcolepsy (Cronbach alpha=0.94) and OSA (Cronbach alpha=0.93). Test-retest reliability was high for both participants with narcolepsy (interclass coefficient=0.94) and OSA (interclass coefficient=0.93).

Conclusion: Interim results suggest the PN-PROS is a valid and reliable measure for the evaluation of symptom frequency and burden for pediatric patients with narcolepsy. Data collection is ongoing, utilizing additional sites in other regions of the United States to ensure generalizability of our findings.