Burden of Pediatric Narcolepsy on Patients and Caregivers

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Introduction

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- Narcolepsy is a rare, chronic sleep disorder with symptoms including excessive daytime sleepiness (EDS), cataplexy, hypnagogic/hypnopompic hallucinations, sleep paralysis, and disrupted nighttime sleep that presents primarily between the ages of 7 and 25^{1,2}
- No real-world studies to date have assessed the burden of narcolepsy on pediatric patients and their caregivers
- To address this need, the Children, Adolescents, and Their providers: the Narcolepsy Assessment Partnership (CATNAP[®], NCT04899947) registry collects data about the burden of pediatric narcolepsy on patients and their caregivers based on participant and caregiver reports of quality of

Severe EDS n=10; 34.5% 80 Clinically Significant of patients 70 EDS 60 (ESS > 10) Moderate EDS n=20; 69.0% n=7; 24.1% ge Percenta 40 Mild EDS n=3; 10.3% 30

Figure 2. Pediatric Narcolepsy Burden on Patients Upon Enrollment: Clinically Significant Excessive Daytime Sleepiness

life, social functioning, and work productivity³

Objective

• To assess the real-world burden of narcolepsy on pediatric patients and their caregivers in a cross-sectional analysis of participants at the time of enrollment in the CATNAP registry

Methods

- CATNAP is a prospective, multicenter registry that is collecting real-world data from 17 clinical sites (starting in September 2020)
- Inclusion criteria for this analysis were children and adolescents with narcolepsy, aged 18 or younger at enrollment, enrolled at clinical sites through February 2023, and with no missing data for selected patientand caregiver-reported outcomes
- Using web-based portals, participants, caregivers, and clinicians completed an initial survey on sociodemographic characteristics; diagnostic and medical history; past or current treatment; comorbidities; and disease progression
- Patient burden was measured by the following:
 - Epworth Sleepiness Scale for Children and Adolescents (ESS-CHAD; higher scores indicate more sleepiness)⁴
 - Pediatric Quality of Life Inventory (PedsQL; higher scores indicate better outcome)⁵
 - Patient-Reported Outcomes Measurement Information System: Peer Relationships (PROMIS; higher scores indicate better relationships)⁶
- Caregiver burden was measured by the following:
 - Work Productivity and Activity Impairment questionnaire (higher) percentages indicate greater impairment and less productivity)⁷
 - Caregiver Well-Being-short form (CWB-sf; higher scores indicate greater well-being)⁸

Specialist physicians who provide treatment to pediatric patients

with narcolepsy invited to participate as a CATNAP registry site

Pediatric patients and their caregivers invited to participate via

patient portal by registry site personnel or treating physician

Eligible participants include any child or adolescent (<18 years of age)

willing and able to participate in an English-based registry

and provide informed consent

Figure 1. CATNAP Study Flow



ESS-CHAD Categorisation

^aEDS categories were defined as follows: Lower Normal, ESS 0–5 (0% of CATNAP participants); Higher Normal, ESS 6–10; Mild EDS, ESS 11–12; Moderate EDS, ESS 13–15; Severe EDS, ESS 16–24. EDS, excessive daytime sleepiness; ESS-CHAD, Epworth Sleepiness Scale for Children and Adolescents.

• The mean (SD) ESS-CHAD score was 14.0 (4.8), corresponding to moderate EDS

Figure 3. Pediatric Narcolepsy Burden on Patients: Low Quality of Life Scores Across Physical, Emotional, Social, and School Domains



^aPedsQL scores range from 0–100, with higher score indicating higher quality of life. ^bTotal score is the average of all items across the 4 quality dimensions. Data captured upon enrollment.

PedsQL, Pediatric Quality of Life Inventory; QoL, quality of life; SD, standard deviation.

• Mean PedsQL scores were below normative values in all domains, with the greatest difference in School Function⁵

Figure 4. Pediatric Narcolepsy Burden on Patients: Impact on Peer Relationships







Collection

Data

Diagnosis verified by registry personnel or an enrolling physician through evidence from medical history and supporting medical documents

Patient, caregiver, and clinician data collected via web-based online portal with surveys

Results

Table 1. Participant Characteristics

Characteristic	Participants (N=29)
Age at enrollment, years	
Mean (SD)	14.3 (3.1)
Median (Q1, Q3)	15.0 (13.0, 16.0)
Min, Max	4.0, 18.0
Sex, n (%)	
Female	13 (44.8)
Male	12 (41.4)
Not available	4 (13.8)
Race, n (%)	
American Indian or Alaska Native	1 (3.4)
Asian	0
Black or African American	11 (37.9)
Native Hawaiian or Other Pacific Islander	1 (3.4)
White	12 (41.4)
Not available	4 (13.8)
Ethnicity, n (%)	
Hispanic or Latino	5 (17.2)
Not Hispanic or Latino	20 (69.0)
Not available	4 (13.8)
Region, n (%)	
Midwest	6 (20.7)
Northeast	6 (20.7)
South	9 (31.0)
West	4 (13.8)
Not available	4 (13.8)
Narcolepsy diagnosis type (clinician reported), n (%)	
Type 1 (with cataplexy)	19 (65.5)
Type 2 (without cataplexy)	5 (17.2)
Not available	5 (17.2)
Narcolepsy diagnosis type (participant reported), n (%)	
Type 1 (with cataplexy)	17 (58.6)
Type 2 (without cataplexy)	7 (24.1)
Not available	5 (17.2)

Note: Data captured upon enrollment.

PROMIS, Patient-Reported Outcomes Measurement Information System: Peer Relationships; SD, standard deviation.

• Mean PROMIS T-score was below the population-standardized mean value, indicating lower quality of peer relationships



Figure 6. Pediatric Narcolepsy Burden on Caregivers: Reduced Ability to Meet Basic Needs and Activities of Daily Living^a





^a19 of 29 caregivers (65.5%) were employed and were presented questions on work productivity. ^bWork Productivity loss reflects both absenteeism and presenteeism. Activity Impairment questions were presented to everyone. Data captured upon enrollment.

SD, standard deviation; WPAI, Work Productivity and Activity Impairment.

• The mean work time missed (absenteeism) was 1.5%, while mean reduction in productivity at work (presenteeism) was 30.0%, and mean impairment of overall activity was 35.0%

CWB-sf, Caregiver Well-Being-short form; SD, standard deviation

Conclusions

- This descriptive study of children, adolescents, and their caregivers in the CATNAP registry illustrates the broad burden of narcolepsy on both groups at enrollment
- Patient-reported scores suggested pathologic sleepiness, impacts to quality of life, and impaired peer relationships; the greatest burden was related to daytime sleepiness and school functioning
- The greatest burden on caregivers was related to work productivity and activity impairment

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Q1, first quartile; Q3, third quartile; SD, standard deviation.

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