

# Burden of Pediatric Narcolepsy on Patients and Caregivers

Deborah A. Nichols, MS<sup>1</sup>; Wayne Macfadden, MD<sup>2</sup>; Eileen B. Leary, PhD, RPSGT<sup>1,\*</sup>; Femida Gwady-Sridhar, PhD, RPH<sup>3</sup>; Judith Owens, MD, MPH<sup>4</sup>

<sup>1</sup>Jazz Pharmaceuticals, Palo Alto, CA, USA; <sup>2</sup>Jazz Pharmaceuticals, Philadelphia, PA, USA; <sup>3</sup>Pulse Inframe, London, Ontario, CA; <sup>4</sup>Boston Children's Hospital, Harvard Medical School, Waltham, MA, USA. \*Elizabeth B. Leary is a former employee of Jazz Pharmaceuticals.

## Introduction

- 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<sup>1,2</sup>
- 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<sup>®</sup>, NCT04899947) registry collects data about the burden of pediatric narcolepsy on patients and their caregivers based on participant and caregiver reports of quality of life, social functioning, and work productivity<sup>3</sup>

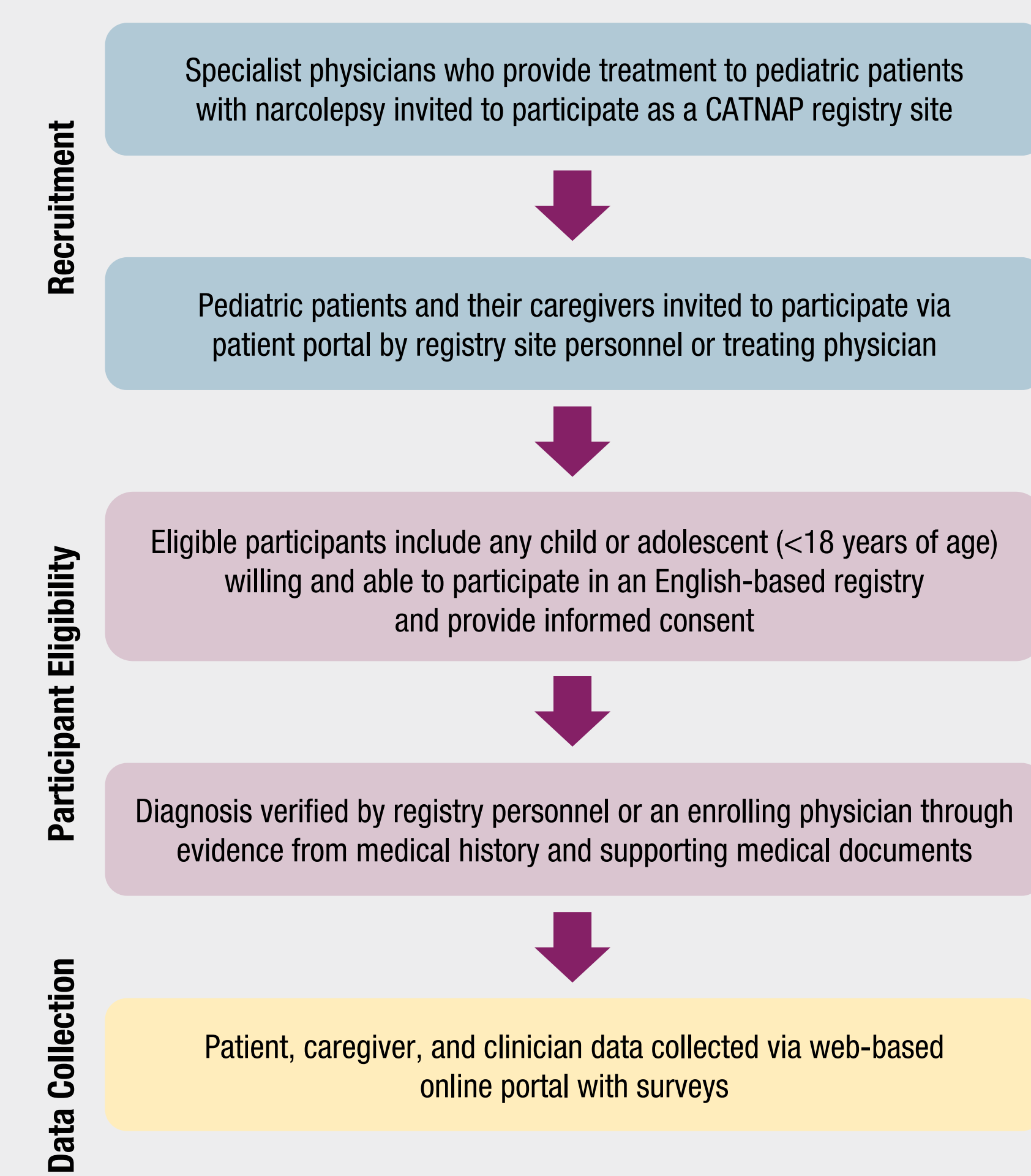
## Objective

- 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 patient- and 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)<sup>4</sup>
  - Pediatric Quality of Life Inventory (PedsQL; higher scores indicate better outcome)<sup>5</sup>
  - Patient-Reported Outcomes Measurement Information System: Peer Relationships (PROMIS; higher scores indicate better relationships)<sup>6</sup>
- Caregiver burden was measured by the following:
  - Work Productivity and Activity Impairment questionnaire (higher percentages indicate greater impairment and less productivity)<sup>7</sup>
  - Caregiver Well-Being—short form (CWB-sf; higher scores indicate greater well-being)<sup>8</sup>

### Figure 1. CATNAP Study Flow



## Results

Table 1. Participant Characteristics

Characteristic	Participants (N=29)
<b>Age at enrollment, years</b>	
Mean (SD)	14.3 (3.1)
Median (Q1, Q3)	15.0 (13.0, 16.0)
Min, Max	4.0, 18.0
<b>Sex, n (%)</b>	
Female	13 (44.8)
Male	12 (41.4)
Not available	4 (13.8)
<b>Race, n (%)</b>	
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)
<b>Ethnicity, n (%)</b>	
Hispanic or Latino	5 (17.2)
Not Hispanic or Latino	20 (69.0)
Not available	4 (13.8)
<b>Region, n (%)</b>	
Midwest	6 (20.7)
Northeast	6 (20.7)
South	9 (31.0)
West	4 (13.8)
Not available	4 (13.8)
<b>Narcolepsy diagnosis type (clinician reported), n (%)</b>	
Type 1 (with cataplexy)	19 (65.5)
Type 2 (without cataplexy)	5 (17.2)
Not available	5 (17.2)
<b>Narcolepsy diagnosis type (participant reported), n (%)</b>	
Type 1 (with cataplexy)	17 (58.6)
Type 2 (without cataplexy)	7 (24.1)
Not available	5 (17.2)

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

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

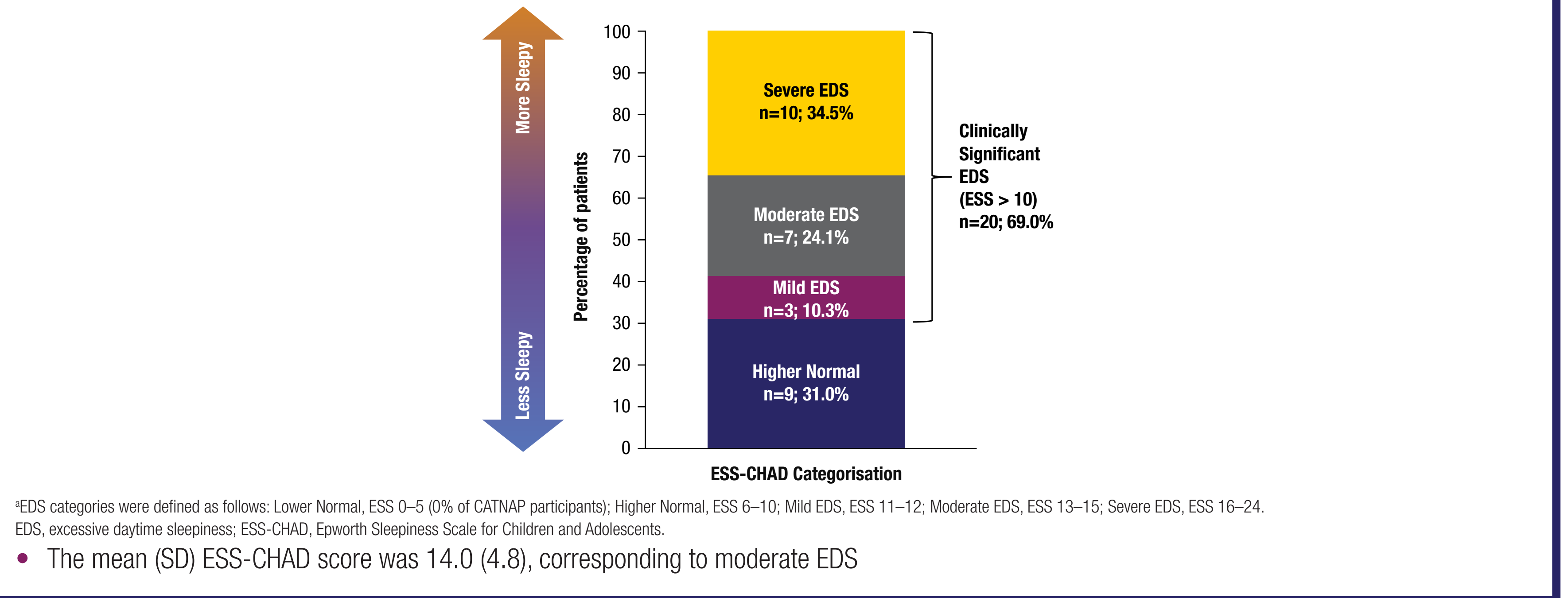


Figure 3. Pediatric Narcolepsy Burden on Patients: Low Quality of Life Scores Across Physical, Emotional, Social, and School Domains

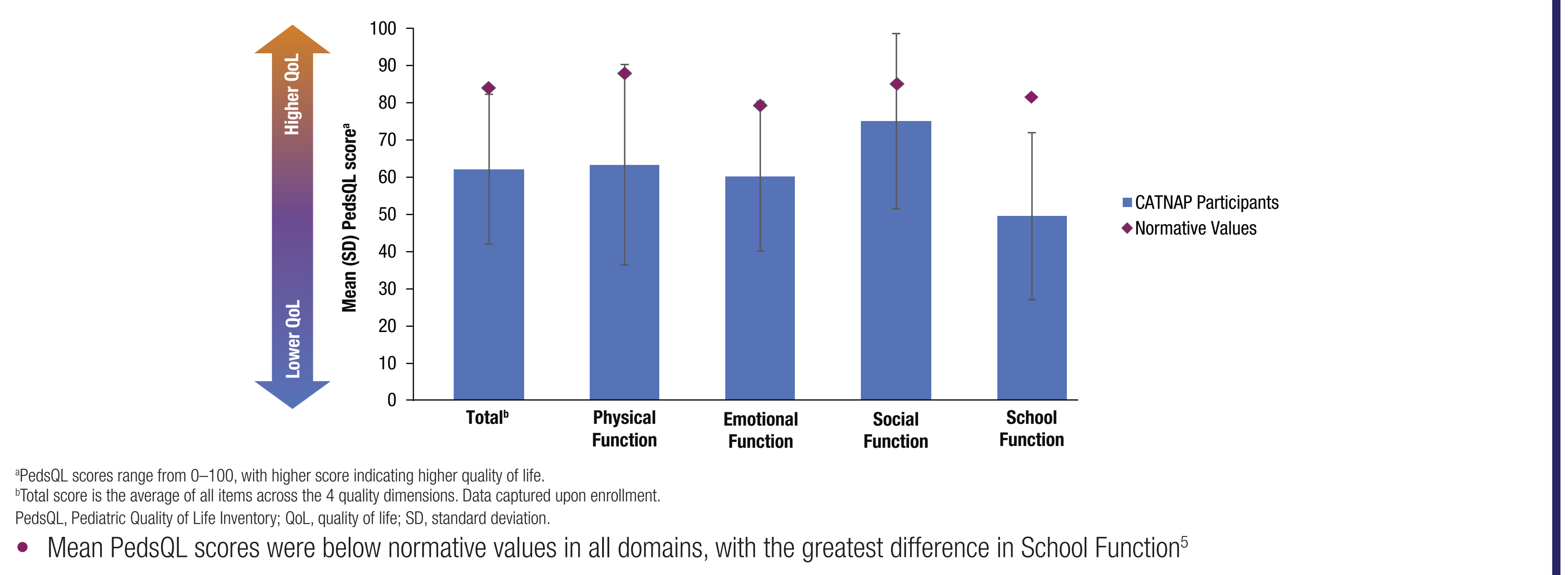


Figure 4. Pediatric Narcolepsy Burden on Patients: Impact on Peer Relationships

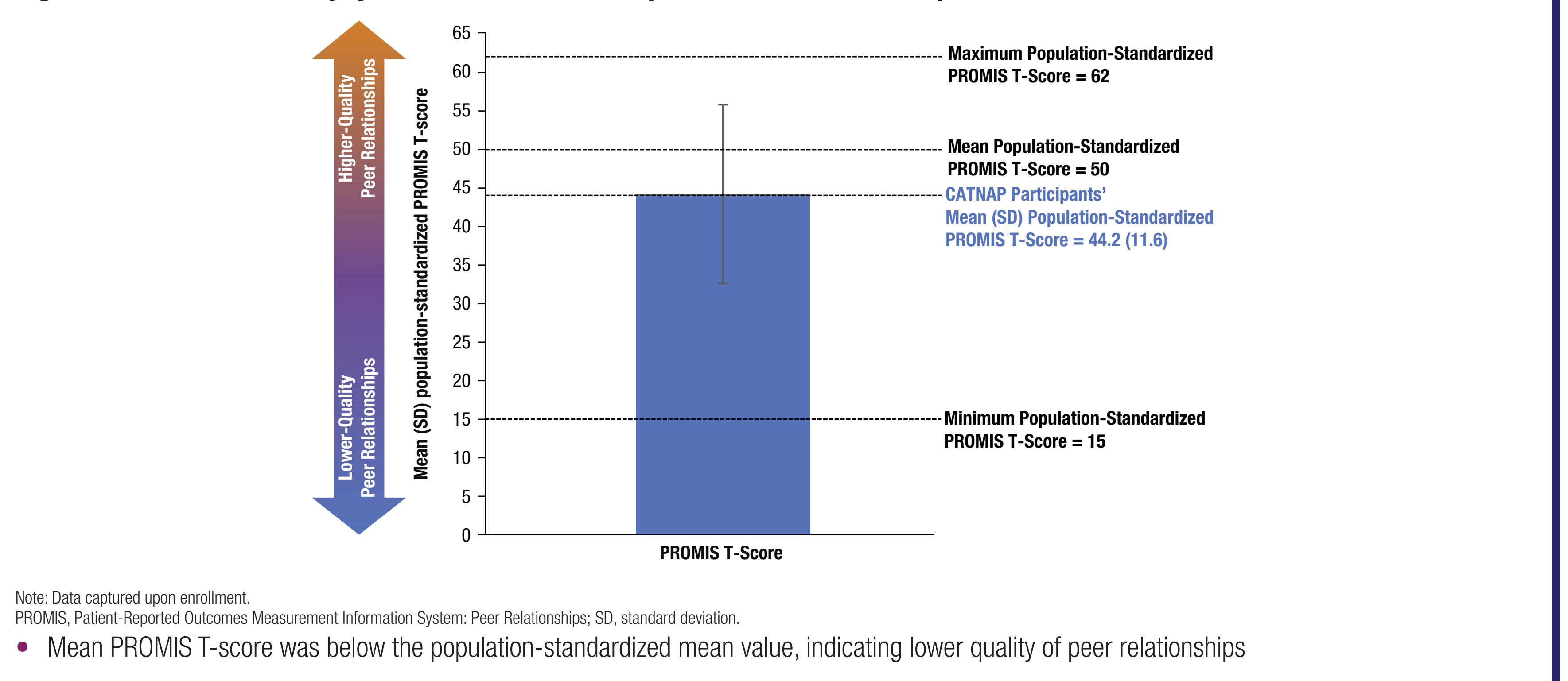


Figure 5. Pediatric Narcolepsy Burden on Caregivers: Impairment in Work Productivity<sup>a</sup> and Overall Activity

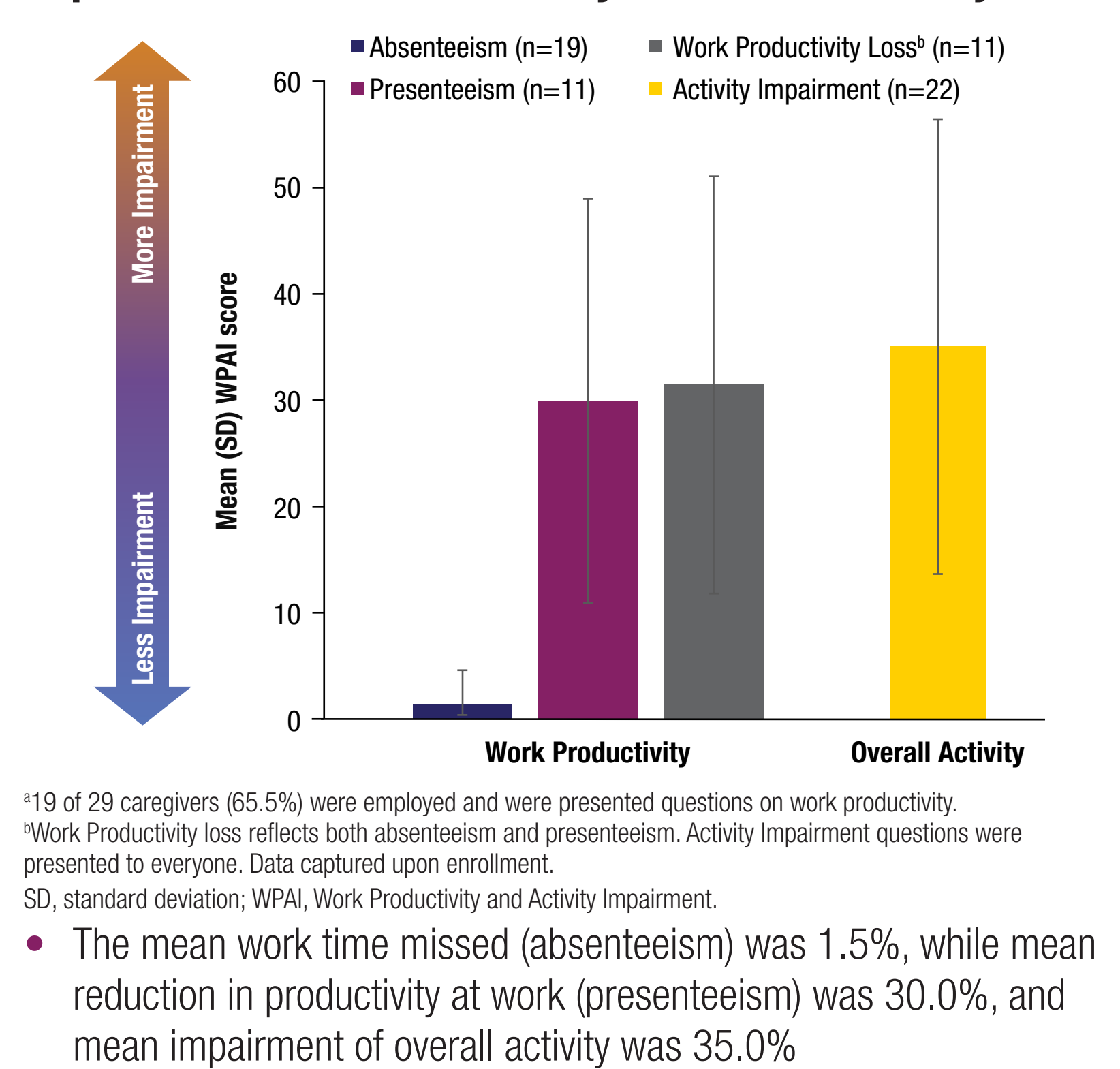
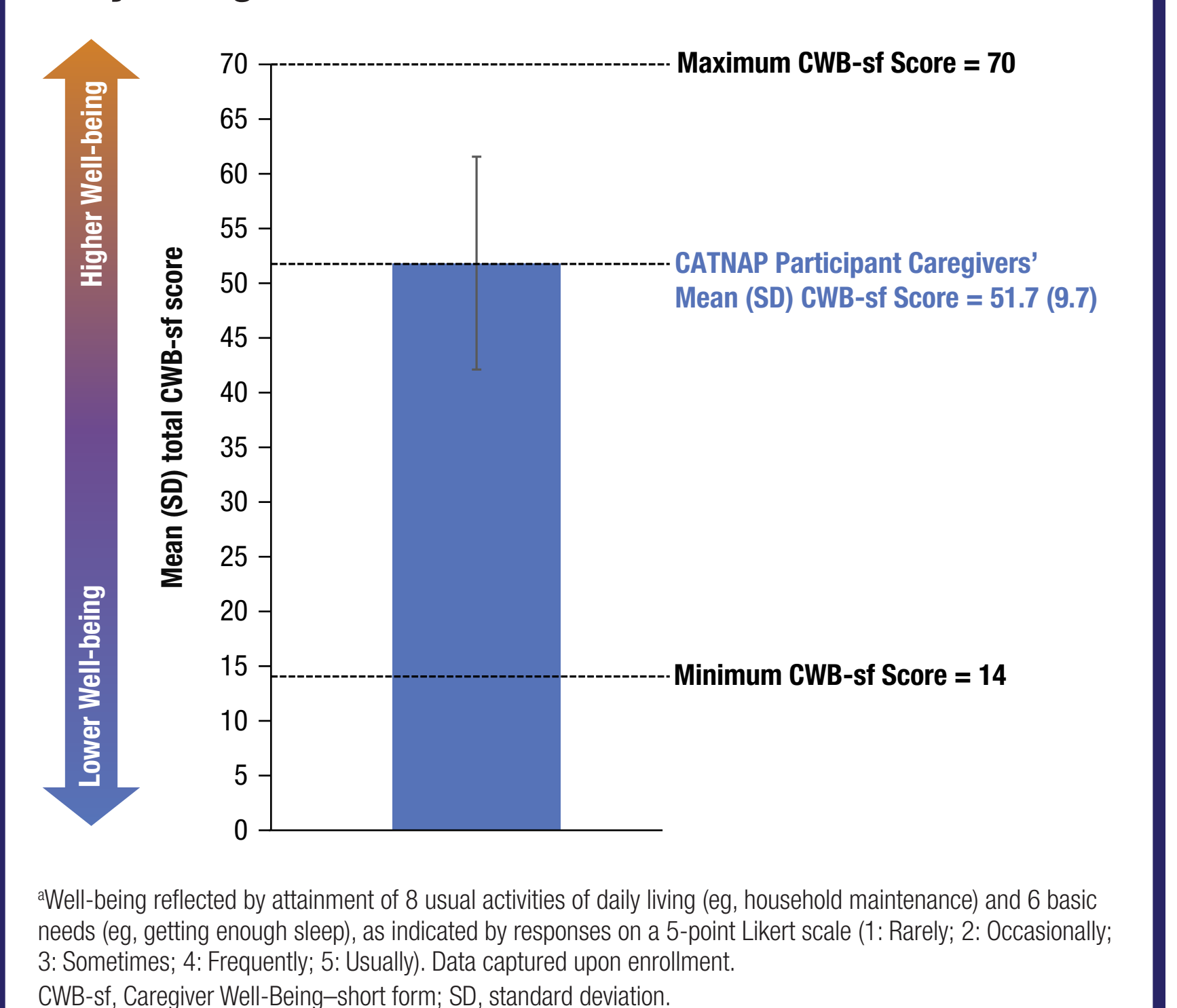


Figure 6. Pediatric Narcolepsy Burden on Caregivers: Reduced Ability to Meet Basic Needs and Activities of Daily Living<sup>a</sup>



## 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

**References:** 1. American Academy of Sleep Medicine. *International Classification of Sleep Disorders—Third Edition, Text Revision* (ICSD-3-TR). Darien, IL: American Academy of Sleep Medicine; 2023. 2. Thorpy MJ, Krieger AC. *Sleep Med*. 2014;15(5):502-7. 3. Nichols DA, Macfadden W, Leary EB, Gwady-Sridhar F, Owens J. Presented at: Annual Scientific Meeting of the Associated Professional Sleep Societies; June 3-7, 2023; Indianapolis, IN. 4. Wang GY, Menno D, Chen A, et al. *Sleep Med*. 2022;89:78-84. 5. Varni JW, Burwinkle TM, Seid M, Skarr D. *Ambul Pediatr*. 2003;3(6):329-41. 6. Dewalt DA, Thissen D, Stucky BD, et al. *Health Psychol*. 2013;32(10):1093-103. 7. Reilly MC, Zbrozek AS, Dukes EM. *Pharmacoeconomics*. 1993;4(5):353-65. 8. Tebb SS, Berg-Weger M, Rubio DM. *Health Soc Work*. 2013;38(4):222-30.

**Support and Acknowledgments:** The authors thank the participants enrolled in the CATNAP registry. This study was supported by Jazz Pharmaceuticals. Data collection and analyses were supported by the Data Science Team (Marc Mason and Angel Cronin) at Pulse Inframe, Inc. Under the direction of the authors, Emilie Croisier, PhD, Shawn Jaramillo, PharmD, and Michael J. Theisen, PhD, of Peloton Advantage, LLC, an OPEN Health company, provided medical writing and editorial support for this poster, which was funded by Jazz Pharmaceuticals.

**Disclosures:** DA Nichols and W Macfadden are full-time employees of Jazz Pharmaceuticals who, in the course of this employment, have received stock options exercisable for, and other stock awards of, ordinary shares of Jazz Pharmaceuticals, plc. EB Leary is a former employee of Jazz Pharmaceuticals who, in the course of this employment, received stock options exercisable for, and other stock awards of, ordinary shares of Jazz Pharmaceuticals, plc. F Gwady-Sridhar is the founder and CEO of Pulse Inframe, Inc., the platform technology company that built the CATNAP registry. J Owens has received consultancy fees from Citrine, Clair Labs, Harmony Biosciences, Idorsia, Jazz Pharmaceuticals, and Sleep Number and receives royalties from Taylor & Francis, WebMD, and Wolters Kluwer.



Scan this code to access this poster online. This code is not for promotional purposes.