

Children, Adolescents, and Their providers: the Narcolepsy Assessment Partnership (CATNAP™) Paediatric Narcolepsy Registry: Study Design

World Sleep 2022
11-16 March 2022
Rome, Italy

Wayne Macfadden, MD¹; Marisa Whalen, PharmD, RPh¹; Teresa L. Steininger, PhD²; Narong Simakajornboon, MD³; Femida Gwadry-Sridhar, BSc Pharm, MSc (Epi), PhD⁴; Judith A. Owens, MD, MPH⁵

¹Jazz Pharmaceuticals, Philadelphia, PA, USA; ²Jazz Pharmaceuticals, Palo Alto, CA, USA; ³Cincinnati Children's Hospital, Cincinnati, OH, USA; ⁴Pulse Inframe, Inc., London, ON, Canada; ⁵Harvard Medical School, Boston Children's Hospital, Waltham, MA, USA

Introduction

- Limited information is available on the natural history, presentation, and management of paediatric narcolepsy
- Children, Adolescents, and Their providers: the Narcolepsy Assessment Partnership (CATNAP™)* is a retrospective and prospective longitudinal, multicentre, web-based paediatric narcolepsy registry (ClinicalTrials.gov identifier: NCT04899947)
- CATNAP is a next-generation registry that collects relevant real-world clinical information from patients, caregivers, and clinicians

Objectives

- The primary objectives are to improve understanding of the natural history of paediatric narcolepsy, characterise symptom presentation and diagnosis in paediatric patients, and understand treatment practices and outcomes
- Secondary objectives are to understand the quality of life of paediatric patients and their caregivers, burden of disease and healthcare resource utilisation of patients, caregiver burden, and impact on patients' social development and academic/educational outcomes

Methods

Table 1. Patient/Caregiver-Entered Data About Patients

	Enrolment	Retrospective/Prospective Data Collection
Family Medical History	X	X
Medical History	X	X
Diagnosis	X	
Diagnostic tests performed		
Disease symptoms		
Patient's disease history		
Previous treatments		
Patient diagnostic journey		
Comorbid Conditions	X	X
Disease Progression	X	X
Daytime sleepiness as measured by ESS-CHAD		
Number of cataplexy attacks per week		
Sleep quality		
Other symptoms		
Treatment History	X	X
Name of treatment		
Daily dose/PRN		
Start, end date		
Rationale for treatment		
If discontinued, rationale for discontinuation		

ESS-CHAD, Epworth Sleepiness Scale for Children and Adolescents; PRN, as needed.

Table 3. Clinician-Entered Data About Patients

	Enrolment	Retrospective/Prospective Data Collection
Diagnosis	X	
Sleep study (PSG, MSLT)		
HLA testing		
CSF hypocretin		
Symptoms at diagnosis		
Anthropometrics	X	X
Height, weight at diagnosis		
Current height, weight		
Comorbid Conditions	X	X
Disease Progression	X	X
Daytime sleepiness as measured by ESS-CHAD		
Number of cataplexy attacks per week		
Sleep quality		
Other symptoms		
Treatment History	X	X
Name of treatment		
Daily dose/PRN		
Start, end date		
Rationale for treatment		
If discontinued, rationale for discontinuation		
Clinical Outcomes	X	X
Medical Records	X	X

CSF, cerebrospinal fluid; ESS-CHAD, Epworth Sleepiness Scale for Children and Adolescents; HLA, human leukocyte antigen; MSLT, Multiple Sleep Latency Test; PRN, as needed; PSG, polysomnography.

Results

- CATNAP enrolment opened in October 2020
- Initial data are anticipated to be presented starting in mid-2022

Methods

Figure 1. Study Flow Diagram

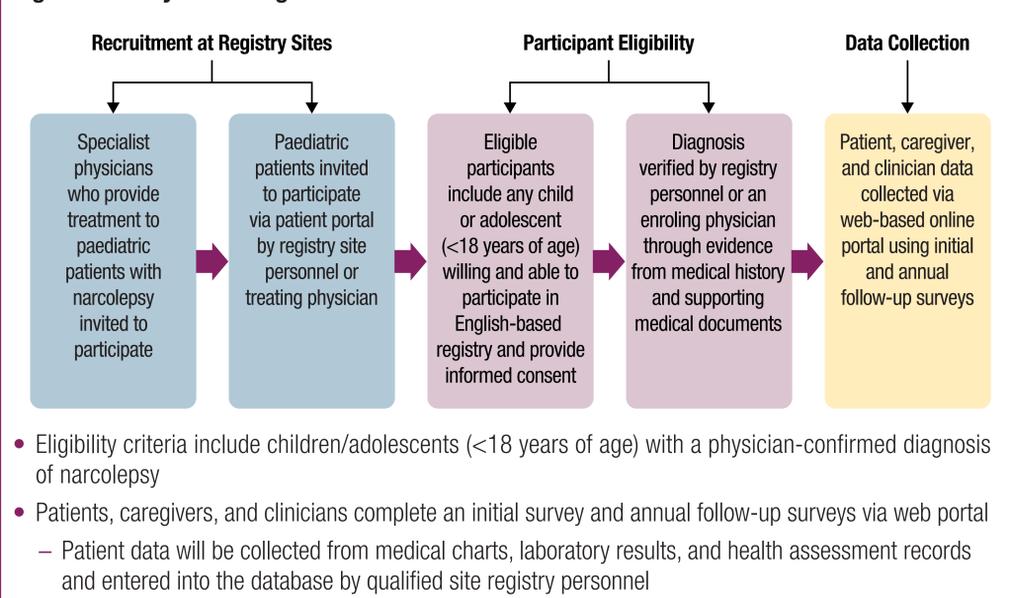


Figure 2. Data Collection Responsibilities

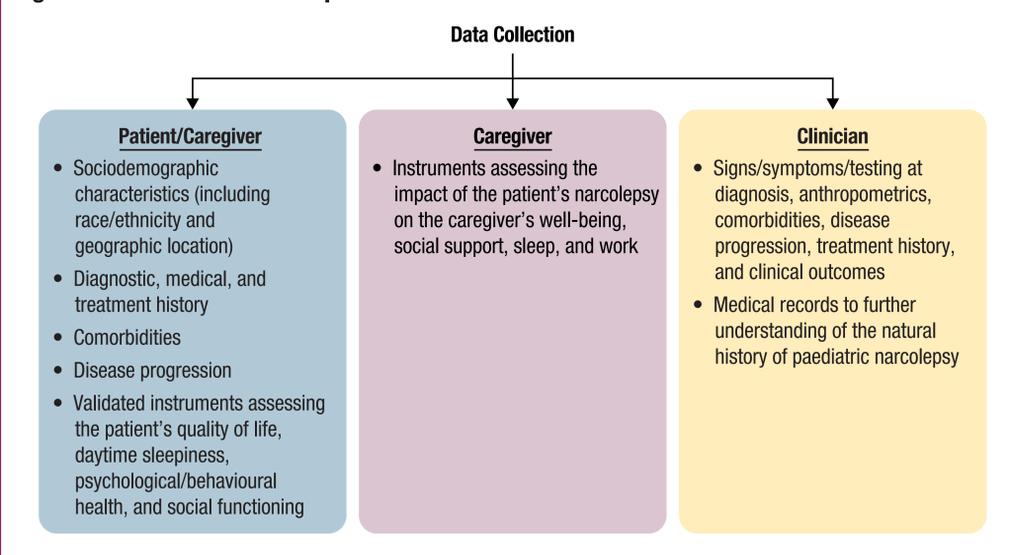


Table 2. Patient/Caregiver- and Caregiver-Entered Symptom and Quality of Life Assessments

PROMs and HRQoL	Initial Completion Schedule	Follow-up Completion Schedule
ESS-CHAD	Enrolment	Annually
PEDsQL	Enrolment	Annually
Child Behaviour Checklist	Enrolment	Annually
PROMIS Peer Relationship-Parent Proxy Short Form v2.0	Enrolment	Annually
Caregiver Work Limitations Questionnaire (C-WLQ) or Work Productivity and Activity Impairment (WPAI) Questionnaire	1 Month after enrolment	None
Caregiver Well-Being Scale (Shortened)	1 Month after enrolment	None
Social Support Survey Instrument	1 Month after enrolment	None
Pittsburgh Sleep Quality Index (PSQI)	1 Month after enrolment	None

ESS-CHAD, Epworth Sleepiness Scale for Children and Adolescents; HRQoL, health-related quality of life; PEDsQL, Pediatric Quality of Life Inventory; PROMIS, Patient-Reported Outcomes Measurement Information System; PROMs, patient-reported outcome measures.

Conclusions

- CATNAP will be the first longitudinal study in paediatric narcolepsy of key determinants of patient management and outcomes, including the patient experience and patient/caregiver burden
- These data will:
 - Provide long-term information not otherwise captured (eg, change in cataplexy or other symptoms over time, emergence of precocious puberty or other relevant diagnoses or symptoms)
 - Facilitate education of patients and caregivers
 - Inform clinical decision-making
 - Potentially contribute to the development of new treatment strategies through a better understanding of the impact of paediatric narcolepsy on patients and their caregivers

Support and Acknowledgments: This study is supported by Jazz Pharmaceuticals. Under the direction of the authors, Judith Bammert Adams, 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: W Macfadden, M Whalen, and TL Steininger 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. N Simakajornboon was the recipient of funding for investigator-initiated, multicentre research on early onset of narcolepsy, sponsored by Jazz Pharmaceuticals. F Gwadry-Sridhar is the CEO of Pulse Inframe, Inc., which was hired by Jazz Pharmaceuticals to build the CATNAP real-world data registry. JA Owens has received consultancy fees from Jazz Pharmaceuticals, Harmony Biosciences, Idorsia, Citrine, Clair Labs, and Sleep Number, and receives royalties from WebMD, Wolters Kluwer, and Taylor & Francis.



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