Hybrid Decentralized Recruitment Approach to a Rare Disease Registry for Pediatric Patients With Narcolepsy: The CATNAP® Registry

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Introduction

- Limited information is available on the natural history, initial presentation, and subsequent management of pediatric narcolepsy
- Children, Adolescents, and Their providers: the Narcolepsy Assessment Partnership (CATNAP®) is a retrospective and prospective, longitudinal, multicenter, web-based pediatric registry with a decentralized site (NCT04899947)
- The CATNAP registry collects relevant real-world clinical information from patients, caregivers, and clinicians, leveraging both a site-based and a decentralized enrollment option for participants

Objective

- The primary objectives of CATNAP are to improve understanding of the natural history of pediatric narcolepsy, describe the initial symptom presentation and diagnosis, and characterize treatment practices and outcomes
- After registry initiation, an innovative decentralized approach was added to adapt to the post-COVID-19 environment and reach additional participants via social media

Methods

- Since registry initiation in September 2020, 17 sites have been activated, including a virtual site launched in August 2022
- Hybrid decentralized recruitment required a new web interface, Institutional Review Board submissions, and a 7-week social media campaign (1 press release, 5 targeted posts) to accompany the virtual site launch
- Eligibility criteria included children/adolescents (<18 years of age) with narcolepsy; participants at physical sites had a physician-confirmed diagnosis
- Using web-based portals, patients and caregivers (and clinicians at physical sites) completed an initial survey on sociodemographic characteristics; diagnostic, medical, and treatment history; comorbidities; and disease progression
- Participants continue to answer follow-up questions annually until they reach 25 years of age or decline to participate

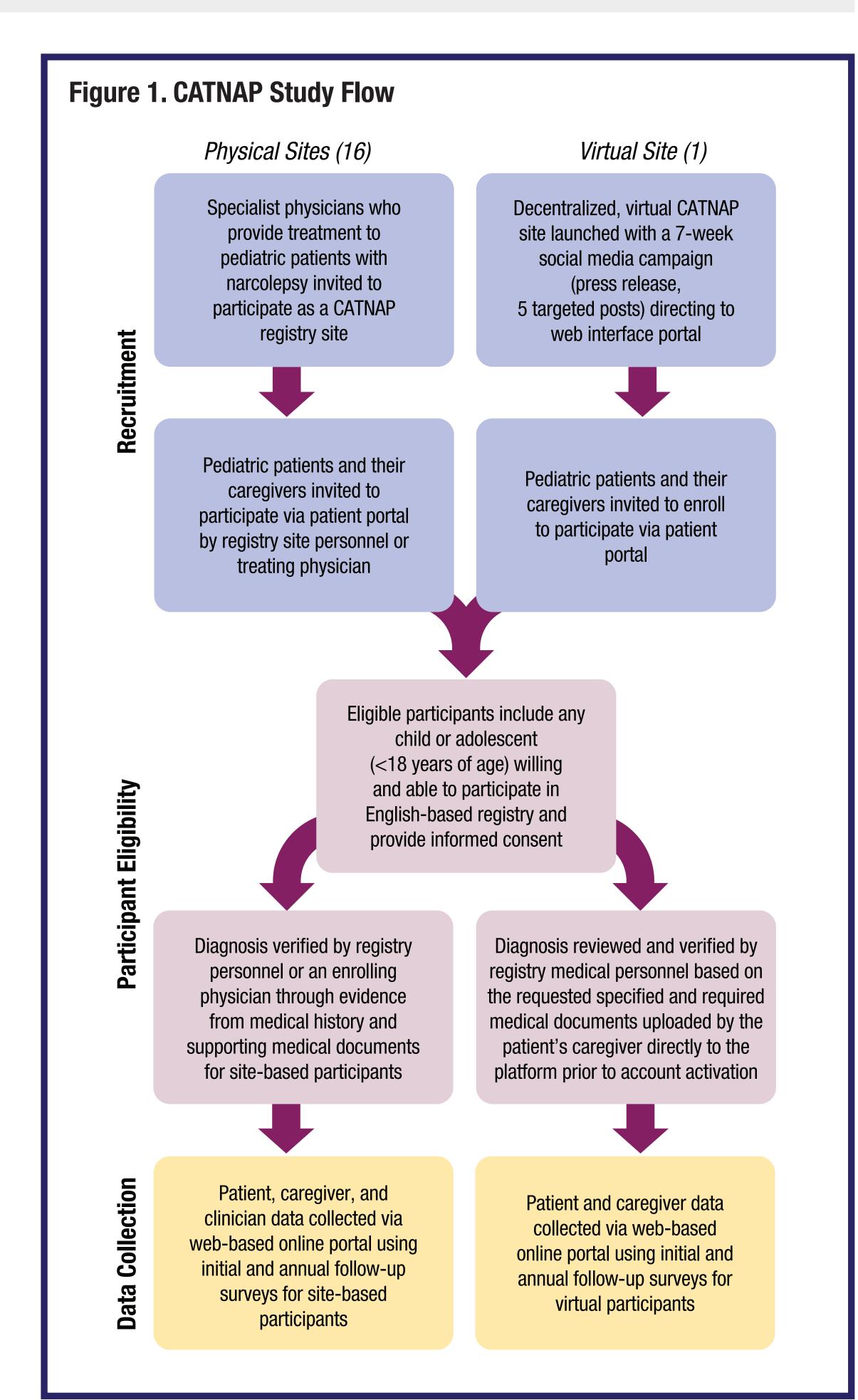


Figure 2. CATNAP Social Media Campaign and Website **Social Media Campaign** Website catna Social media post #3 Social media post #4 Social media post #5 Virtual site launched Social media post #1 (all channels) Newsletter post and summary Newsletter post and summary Newsletter summary Resources This web-based pediatric narcolepsy registry is an organized collection of data focused on health and quality of life information for children under the age of 18 and their aregivers. The data collected will be used to help researchers and physicians improve treatments and outcomes as well better inform the management of this condition fo Select here to learn more Recruiting Now Children's Hospital Study Are you a parent of a child under 18 CATNAP® Pediatric Narcolepsy Registry with narcolepsy and USA based? disorders can impact every facet of CATNAP¹⁷ Pediatric Narcolepsy Registry -Do you want to share your story to help based families with children under ENROLL • @JazzPharma are inviting you to take 18 living with narcolepsy to take part children, adolescents, and their providers: the narcolepsy ATNAPTM Pediatric Narcolepsy Registry part in their patient registry. Help@JazzPharma with their patient researd Does your child have narcolepsy? link to registry: Are you based in the USA? assessment partnership New Institutional Review Board (IRB) #Narcolepsy #PediatricNarcolepsy #Narcolepsy #PediatricNarcolepsy #Narcolepsy #PediatricNarcolepsy of children aged 0-18 years who live with @JazzPharma are inviting Parents/car #SleepDisorders #Sleep narcolepsy in the USA to take part in their of children aged 0-18 years who live with Registry from August 2022 up until November 28, 2022 please #Narcolepsy #PediatricNarcolepsy #Narcolepsy #PediatricNarcoleps note that the IRB of record has About Pediatric Narcoleps For more details please click Narcolepsy is a chronic condition where the brain is not To register and join the CATNAP®: Pediatric Narcolepsy **Parents of Children with** Registry select 'Enroll'. The Institutional Review Board Narcolepsy Invited to approves all documents ie. **Participate in Registry** protocol and consents related to Enroll Remote Participation Model Allows People to Enrol Read More Read More **Jazz Pharmaceuticals Debut** catna About healthie™ 2.0 **Narcolepsy Registry** Privacy and Security Who Can Join? **Contact Information** Patient privacy and data Anyone who is younger than 18 years with a E. catnap@pulseinfoframe.com security are of paramount diagnosis of narcolepsy. Child/Caregivers eligible f y to be compensated \$75 for completing surveys. accelerate research and drug Disclaimer: The content on this site is not meant to replace a conversation with a sleep specialist. A sleep specialist can evaluate your symptoms and make a diagnosis development for Rare Diseases Cancer and Chronic Conditions

Results

Characteristic	Physical Sites (n=74)	Virtual Site (n=51)	Total Participants (N=125)	
Age at enrollment, years		`		
Mean (SD)	14.7 (3.13)	9.9 (2.01)	12.7 (3.59)	
Median (Q1, Q3)	16 (13, 17)	9 (8, 11)	12.7 (3.59)	
Min, max	4, 19	7, 16	4, 19	
Sex, n (%)				
Male	20 (27.0)	24 (47.1)	44 (35.2)	
Female	25 (33.8)	18 (35.3)	43 (34.4)	
Not available	29 (39.2)	9 (17.6)	38 (30.4)	
Race, n (%)				
Asian	0 (0)	1 (2.0)	1 (0.8)	
Black or African American	21 (28.4)	0 (0)	21 (16.8)	
Native Hawaiian or Other Pacific Islander	1 (1.4)	0 (0)	1 (0.8)	
White	23 (31.1)	41 (80.4)	64 (51.2)	
Not available	29 (39.2)	9 (17.6)	38 (30.4)	
Ethnicity, n (%)				
Not Hispanic or Latino	37 (50.0)	42 (82.4)	79 (63.2)	
Hispanic or Latino	8 (10.8)	0 (0)	8 (6.4)	
Not available	29 (39.2)	9 (17.6)	38 (30.4)	
Narcolepsy type, n (%)				
Type 1 (with cataplexy)	52 (70.3)	14 (27.5)	66 (52.8)	
Type 2 (without cataplexy)	11 (14.9)	1 (2.0)	12 (9.6)	
Unsure	11 (14.9)	36 (70.6)	47 (37.6)	
Region, n (%)				
South	18 (24.3)	12 (23.5)	30 (24.0)	
Northeast	14 (18.9)	11 (21.6)	25 (20.0)	
West	3 (4.1)	10 (19.6)	13 (10.4)	
Midwest	10 (13.5)	9 (17.6)	19 (15.2)	
Not available	29 (39.2)	9 (17.6)	38 (30.4)	

Table 2. Social Media Engagement Rates

Table 2. Journal Media Eligagement Hates								
	Facebook (organic)	Facebook (sponsored)	Instagram	LinkedIn	Twitter	Total		
Engagements	13	29	45	54	26	167		
Impressions	1076	12,551	1406	748	1094	16,875		
Engagement rate	1.2%	0.2%	3.2%	7.2%	2.4%	2.8%		
Clicks	10	29	N/A	4	7	50		
Number of posts in period	6	1	6	6	6	25		

- Note: Good engagement rates were considered 3%, 2%, 2%, and 1% for Instagram, Facebook, LinkedIn, and Twitter, respectively.
- Rates of engagement (eg, clicks, likes, and shares) for Instagram (3.2%), LinkedIn (7.2%), and Twitter (2.4%) exceeded "good engagement rate" benchmarks for these platforms
- Although the engagement rate for Facebook was below benchmarks (<2%), the absolute number of "clicks" (29) was the largest among social media platforms in the campaign

Figure 3. Enrollment After Study Site Opening **Total Enrollment** Start of virtual site enrollment Virtual site enrollment n=51 Start o Total - enrollmen enrollment N = 125Physical site 40 enrollment n=74 **Study month** Details of Virtual-Site Enrollment and Social Media Campaign Newsletter posts Newsletter summary Social media post #5 (all channels) Newsletter summary Social media post #4 (all channels) Newsletter post and summary Social media post #3 (all channels) Newsletter feature and posts Press release Social media post #2 (all channels) Newsletter post and summary Virtual site launched Social media post #1 (all channels) Newsletter post and summary Study month

Conclusions

- A hybrid recruitment approach for CATNAP demonstrates the incorporation of patientdriven considerations into clinical trial design and increases patient access to real-world data registries and studies, which is especially important for research in rare diseases
- A targeted social media campaign raises awareness, drives traffic to the registry portal, and results in registry enrollments
- Decentralization can contribute to enrollment in real-world data registries and studies; however, new approaches to encourage ethnic and racial diversity need further exploration
- A successful implementation of a hybrid approach with both physical and virtual opportunities for enrollment may inform designs of future registries and clinical studies

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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 full-time 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 Gwadry-Sridhar** is the founder and CEO of Pulse Infoframe, Inc., the platform technology company that built the CATNAP registry. **J Owens** has received consultancy fees from Jazz Pharmaceuticals, Harmony Biosciences, Idorsia, Citrine, Clair Labs, and Sleep Number, and Taylor & Francis.