

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

Deborah A. Nichols, MS¹; Wayne Macfadden, MD²; Eileen B. Leary, PhD, RPSGT³; Femida Gwadry-Sridhar, RPh, PhD⁴; Judith Owens, MD, MPH⁵

¹Jazz Pharmaceuticals, Palo Alto, CA, USA; ²Jazz Pharmaceuticals, Philadelphia, PA, USA; ³Formerly Jazz Pharmaceuticals, Palo Alto, CA, 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, 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 1. CATNAP Study Flow

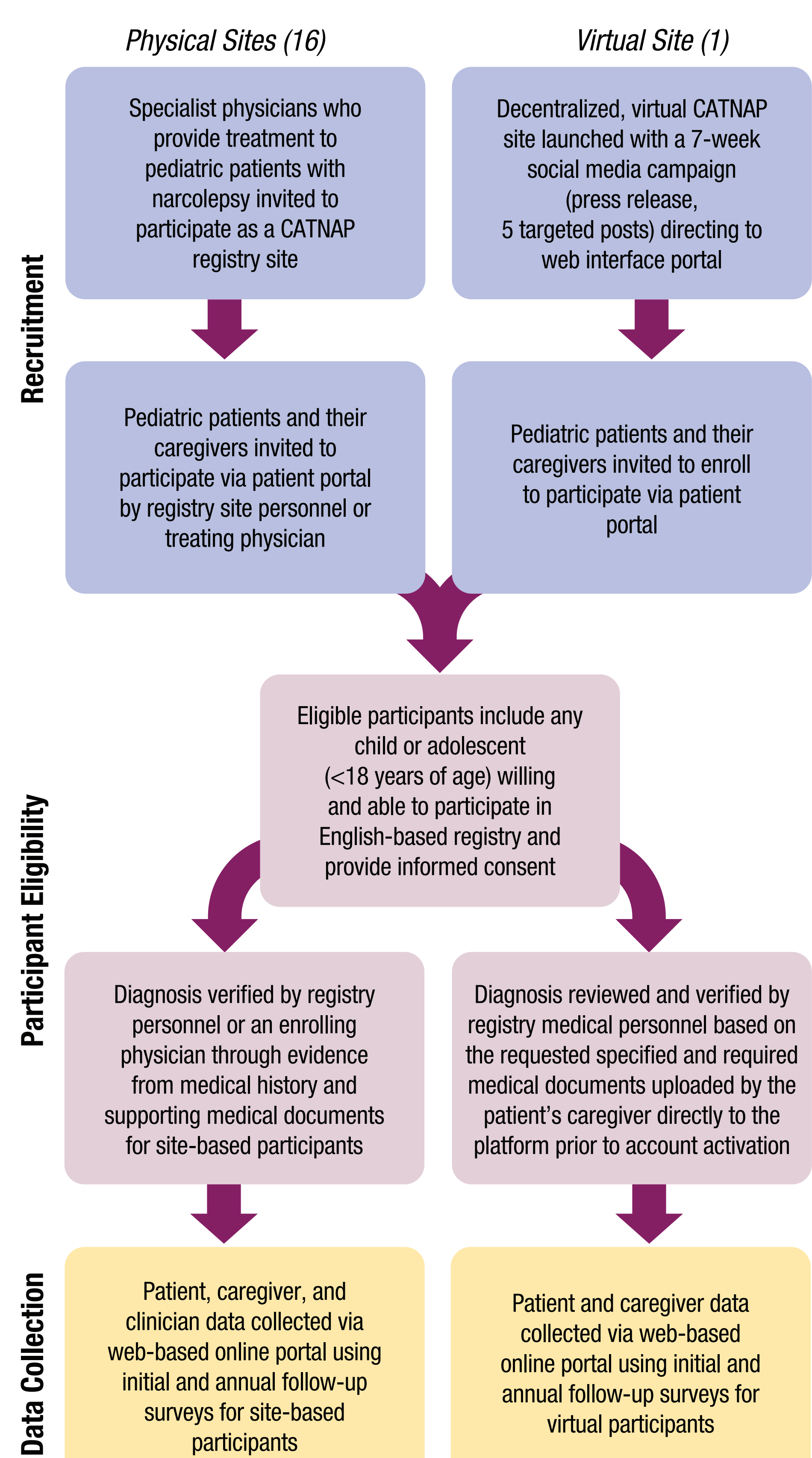
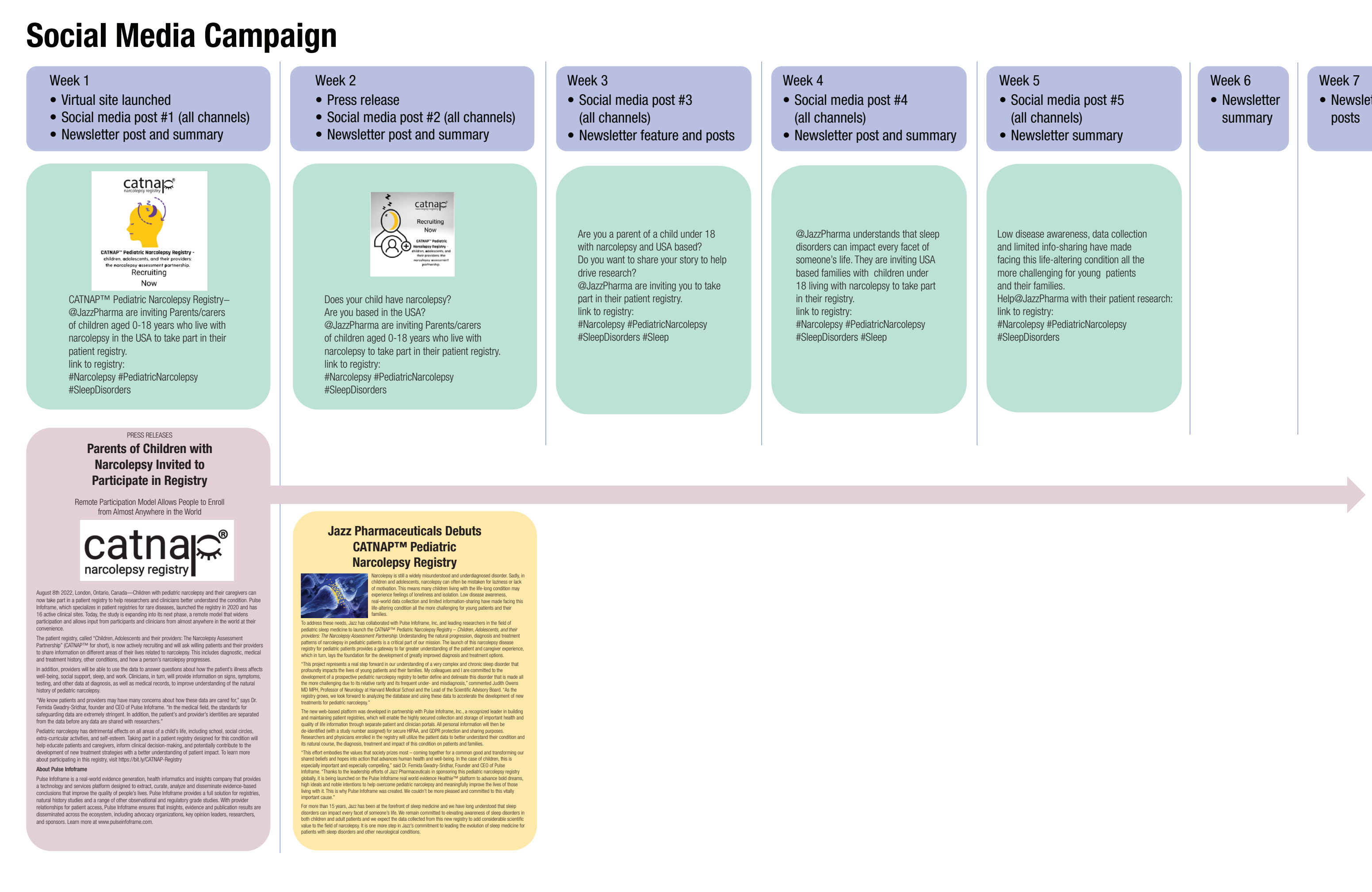


Figure 2. CATNAP Social Media Campaign and Website



Website

Results

Table 1. Baseline Demographics and Disease Characteristics

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)

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

- Patient/caregiver-reported interim baseline data are included for 125 participants

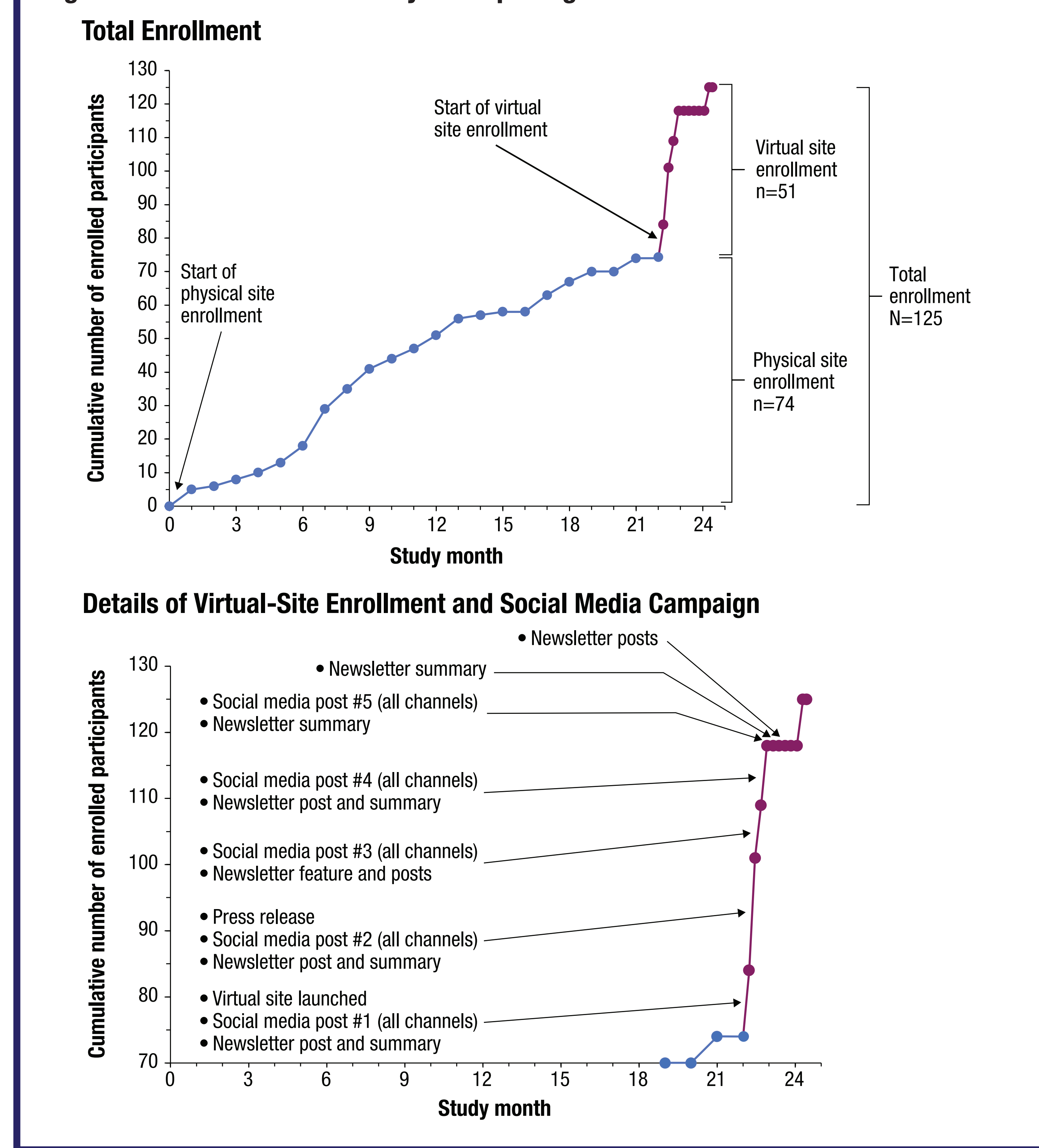
Table 2. Social Media Engagement Rates

	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



Conclusions

- A hybrid recruitment approach for CATNAP demonstrates the incorporation of patient-driven 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 Inframe, 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 receives royalties from WebMD, Wolters Kluwer, and Taylor & Francis.