

Burden of Narcolepsy: A Survey of Patients and Physicians

Michael J. Thorpy, MB, ChB¹; Joseph Hopper, PhD²; Albena Patroneva, MD³

¹Sleep-Wake Disorders Center, Montefiore Medical Center, Bronx, NY, USA; ²Versta Research, Evanston, IL, USA; ³Harmony Biosciences, LLC, Plymouth Meeting, PA, USA

Introduction

- Narcolepsy is a rare neurological disorder that is often under-recognized and misdiagnosed¹
- Narcolepsy is associated with substantial personal and socioeconomic burden on affected patients²⁻⁴
- Surveys of key stakeholders (eg, patients, healthcare providers) can identify unmet needs in medical diagnosis and treatment and highlight areas for improvement in diagnosis, communication between patients and physicians, and ways to improve the lives of people living with narcolepsy

Aim

- To evaluate the perceptions of patients and physicians regarding the burden, symptoms, and treatment of narcolepsy

Methods

- Separate, but coordinated, online surveys were conducted of patients with narcolepsy and treating physicians
 - Respondents were recruited from large national research panels used exclusively for research
- The patient survey was conducted from March 19, 2018, to April 6, 2018, and targeted adult patients who had been previously diagnosed with narcolepsy
- The physician survey was conducted from August 6, 2018, to August 16, 2018, and targeted currently practicing physicians who had treated patients with narcolepsy within the previous 2 years

Results

Survey Respondents

- Survey respondents included 200 patients (Table 1) and 251 physicians (Table 2; Table 3)
 - The patients with narcolepsy surveyed were not necessarily patients of the physicians surveyed
- The percentage of patients with narcolepsy type 1 (38.5%, as diagnosed by physicians) is lower than expected based on prevalence data for narcolepsy with versus without cataplexy^{5,6}

Table 1: Patient Characteristics

Characteristics	Patients (N=200)
Age, y, mean (SD)	46.5 (15.1)
Sex, n (%) [*]	
Male	60 (30.0)
Female	138 (69.0)
Race/ethnicity, n (%) [†]	
White, not Hispanic	158 (79.4)
Black/African American	21 (10.6)
Hispanic	8 (4.0)
Other	12 (6.0)
Narcolepsy diagnosis, n (%)	
Type 1 (with cataplexy)	52 (26.0)
Type 2 (without cataplexy)	89 (44.5)
Unsure	59 (29.5)

^{*}Two patients reported sex as transgender.
[†]n=199.

Table 2: Physician Characteristics

Characteristics	Physicians (N=251)
Years in practice Mean (SD)	19.4 (8.7)
Number of narcolepsy patients per month [*]	
Median	4.0
0–1 patient, n (%)	58 (23.2)
2–4 patients, n (%)	73 (29.2)
5–9 patients, n (%)	36 (14.4)
10–19 patients, n (%)	46 (18.4)
≥20 patients, n (%)	37 (14.8)
Narcolepsy diagnosis, mean percentage of patients [†]	
Type 1 (with cataplexy)	38.5
Type 2 (without cataplexy)	61.5

^{*}n=250.
[†]n=234.

Table 3: Physician Specialty

Specialty, n (%)	Physicians Surveyed (N=251) [*]	Type of Physician Reported by Patients for Most of Their Narcolepsy Care (N=200) [†]
Primary care	69 (27.5)	73 (36.5)
Neurology	66 (26.3)	41 (20.5)
Sleep medicine	46 (18.3)	38 (19.0)
Psychiatry	40 (15.9)	18 (9.0)
Pulmonology	22 (8.8)	16 (8.0)
Board-certified sleep specialist	113 (45.0)	98 (49.0)

^{*}Includes "other" (n=8).
[†]Includes "other" (n=2) and "none" (n=12).

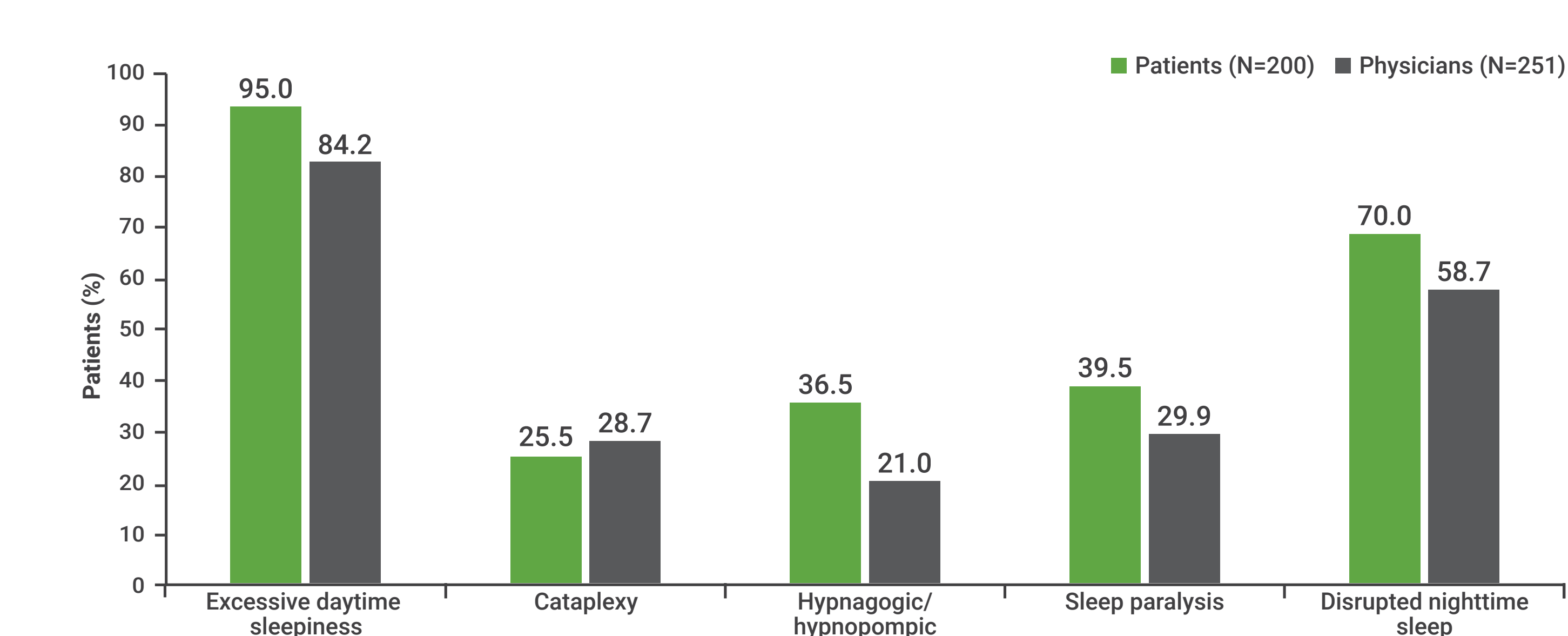
Diagnosis

- Based on patient reports, the average time between symptom onset and narcolepsy diagnosis was 6.4 years
- Physicians estimated that half of all patients with narcolepsy (54.2%) were initially misdiagnosed; in the patient survey, 38.5% reported that they had been misdiagnosed
 - Among patients who had been incorrectly diagnosed, the most common misdiagnoses were depression (24.7%), sleep apnea (16.9%), and insomnia (10.4%)

Symptoms

- One in four patients (25.5%) reported cataplexy ("when a strong positive emotion causes all or part of your body to go weak") as a symptom (Figure 1)
 - An additional 32.5% of patients reported experiencing brief/mild muscular weakness triggered by emotions
 - More than half of patients (53.5%) reported knowing little or nothing about cataplexy
- The percentage of patients with cataplexy, as identified by patients (25.5%) and physicians (28.7%), is much lower than previously reported in the literature (68%)²
- Most patients (87.5%) and physicians (92.0%) identified excessive daytime sleepiness as one of the most disruptive narcolepsy symptoms; however, only 12.5% of all patients (narcolepsy type 1 and narcolepsy type 2) identified cataplexy as a disruptive symptom compared with 70.5% of physicians

Figure 1: Narcolepsy Symptoms

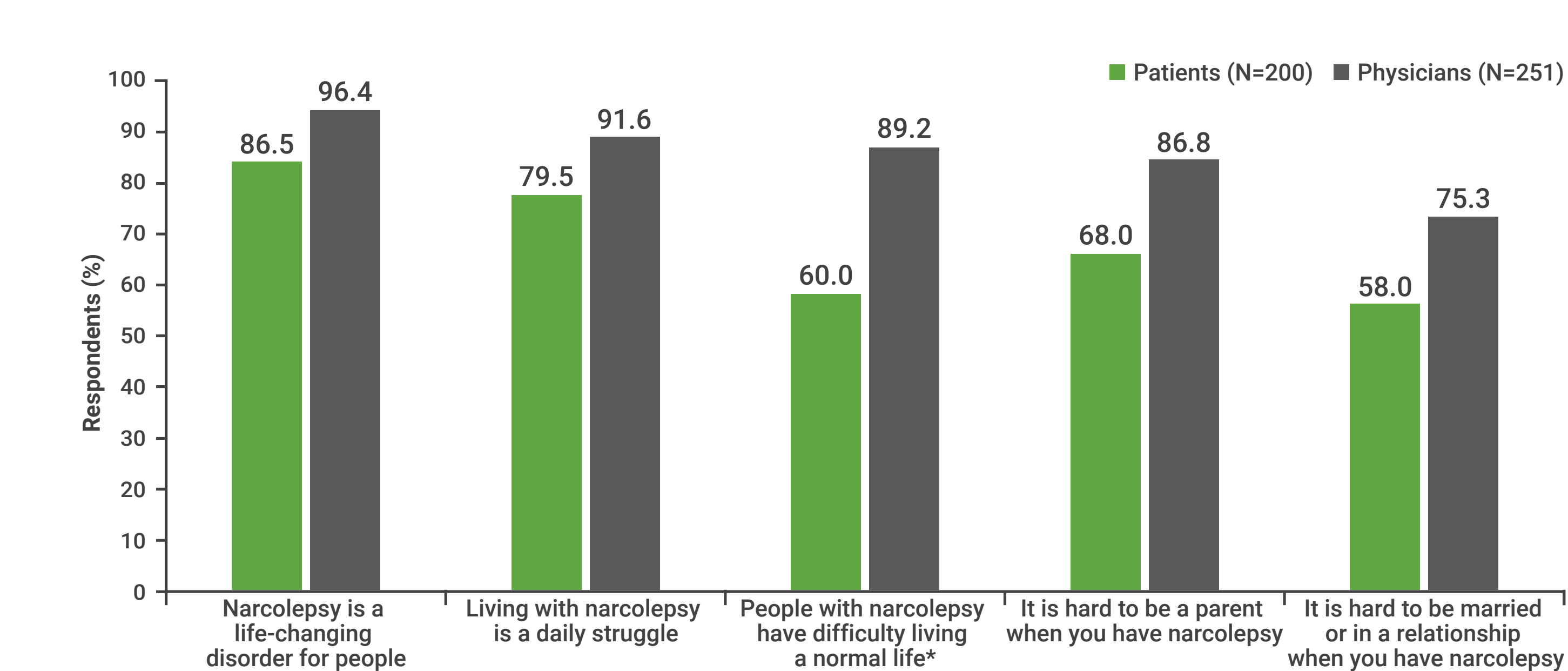


Patient survey: patients with each symptom (%).
Physician survey: percentage of patients with each symptom (mean).

Burden

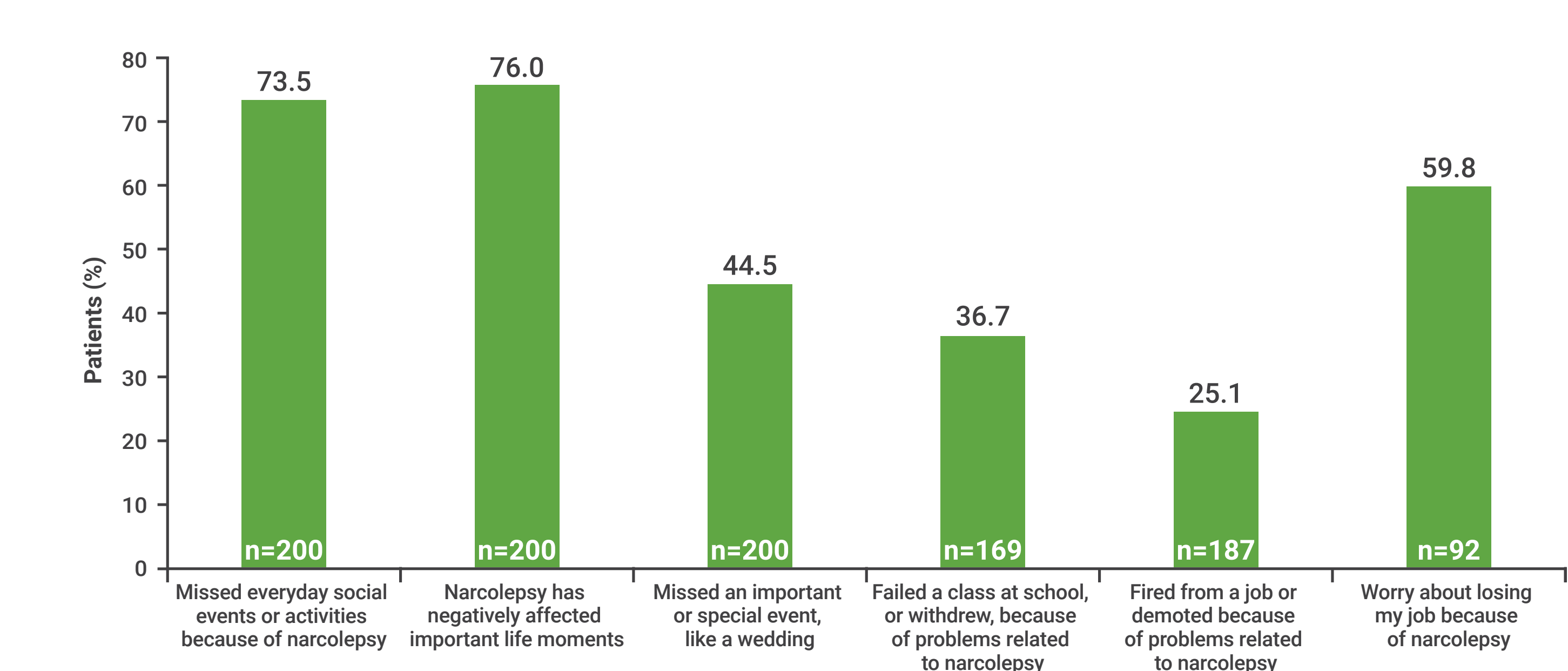
- The impact of narcolepsy can be substantial (Figure 2), affecting many aspects of patients' lives (Figure 3)

Figure 2: Challenges Associated With Narcolepsy



Both surveys: percentage of respondents who strongly agree or somewhat agree.
^{*}Wording of the patient survey item: "I can't live a normal life with narcolepsy."

Figure 3: Additional Challenges: Patient Survey

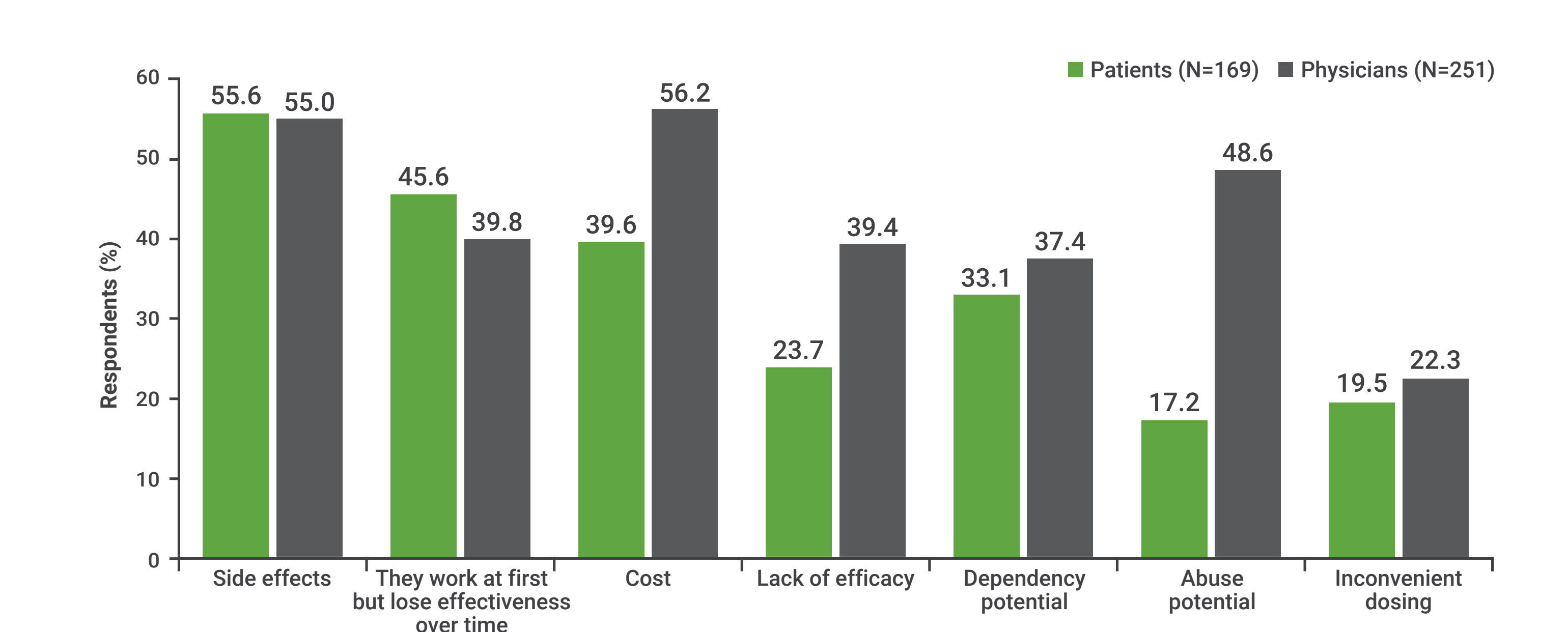


- Narcolepsy also has an emotional impact, with patients reporting that they often feel frustrated (41.5%), anxious (33.0%), depressed (33.0%), embarrassed (28.0%), and angry (23.0%)
- Despite the negative impacts identified by patients, many of them rarely or never discuss with healthcare providers how narcolepsy affects their daily lives (39.9%) or how it affects them emotionally (50.0%)
- Nearly all physicians (93.6%) believe that people with narcolepsy unknowingly alter their lives to accommodate their symptoms
 - Patients report avoiding social interactions and social situations (40.0%) and trying to avoid strong emotions (20.0%)

Treatment

- Physicians reported that they most commonly prescribed wake-promoting agents (to 48.6% of patients, on average), stimulants (28.0%), antidepressants (19.5%), and sodium oxybate (12.4%)
- Patients reported taking stimulants (41.0%), wake-promoting agents (40.5%), antidepressants (35.0%), and sodium oxybate (5.0%)
- Issues associated with narcolepsy medications include adverse events and lack of efficacy (Figure 4)
 - Physicians reported that symptoms were completely or mostly under control in 27.5% of patients, on average, compared with only 12.0% of patients who reported this level of symptom control

Figure 4: Challenges Associated With Narcolepsy Medications



- Two of five physicians (41.8%) said that they are reluctant to prescribe scheduled medications for narcolepsy
- Nearly all patients (94.0%) and physicians (94.0%) said that there is a need for new and better treatment options for narcolepsy
- Nearly all physicians (94.8%) reported that medications offering new mechanisms of action would be valuable to them

Conclusions

- Narcolepsy imposes substantial burden on many aspects of life including school, work, social activities, and relationships
- Physicians may overestimate the degree to which patients' symptoms are controlled
- The relatively low prevalence of cataplexy reported in this survey indicates a need for increased patient education and improved healthcare provider-patient communication about this important symptom
- People with narcolepsy may avoid social situations or emotions in order to avoid cataplexy attacks
- Almost all patients and physicians agreed that there is a need for new, effective treatment options for the management of narcolepsy

REFERENCES

- Thorpy MJ, Morse AM. *Sleep Med Clin*. 2017;12(1):61-71.
- Maski K, et al. *J Clin Sleep Med*. 2017;13(3):419-425.
- Flores NM, et al. *J Clin Sleep Med*. 2016;12(3):401-407.
- Black J, et al. *Sleep Med*. 2014;15(5):522-529.
- Silber MH, et al. *Sleep*. 2002;25(2):197-202.
- Longstreth WT Jr, et al. *Sleep Med*. 2009;10(4):422-426.

ACKNOWLEDGMENTS

This study was funded by Harmony Biosciences, LLC. Technical editorial and medical writing assistance were provided under the direction of the authors by Synchrony Medical Communications, LLC, West Chester, PA, and funded by Harmony Biosciences, LLC, Plymouth Meeting, PA.

DISCLOSURES

MJT reports serving on advisory boards for and receiving research support from Axsome, Balance Therapeutics, Flamel/Avadel, Harmony Biosciences, and Jazz Pharmaceuticals. JH reports being president of Versta Research. AP reports being an employee of Harmony Biosciences, LLC.