

# CRESCENDO: Results From a Survey of Symptom Burden and Quality of Life in Patients with Narcolepsy Type 1

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## Key Question

- What is the patient journey, treatment experience, and unmet need in patients with narcolepsy type 1?

## Conclusions

- The CRESCENDO survey provides valuable patient-generated insights into the burden of illness in persons with NT1
- Despite ongoing treatment, survey respondents reported a wide range of persistent symptoms including cataplexy, EDS, and impaired cognition
- Breakthrough cataplexy was reported by most respondents, substantially impacting their quality of life
- The majority of respondents reported impacts on their work, home, and social lives from cognitive complaints and depressive symptoms
- These results highlight the significant unmet treatment needs of this patient population

## References

- Swick TJ. *Nat Sci Sleep*. 2015; 7: 159–169
- American Academy of Sleep Medicine. ICSD-3-TR. Chicago, IL: 2023.
- España RA, Scammell TE. *Sleep*. 2011;34(7):845-858.
- Tadrous R, et al. *J Sleep Res*. 2021 Dec;30(6):e13383
- Thorpy MJ, Hiller G. *Am Health Drug Benefits*. 2017 Jul;10(5):233-241.
- Krahn LE, et al. *Adv Ther*. 2022; 39(1): 221-243

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## Disclosures

MJT serves as a consultant to Axsome Therapeutics. AK and KH are affiliated with Narcolepsy Network. EBL, SF, and HT are current employees of Axsome Therapeutics. GP is a former employee of Axsome Therapeutics.



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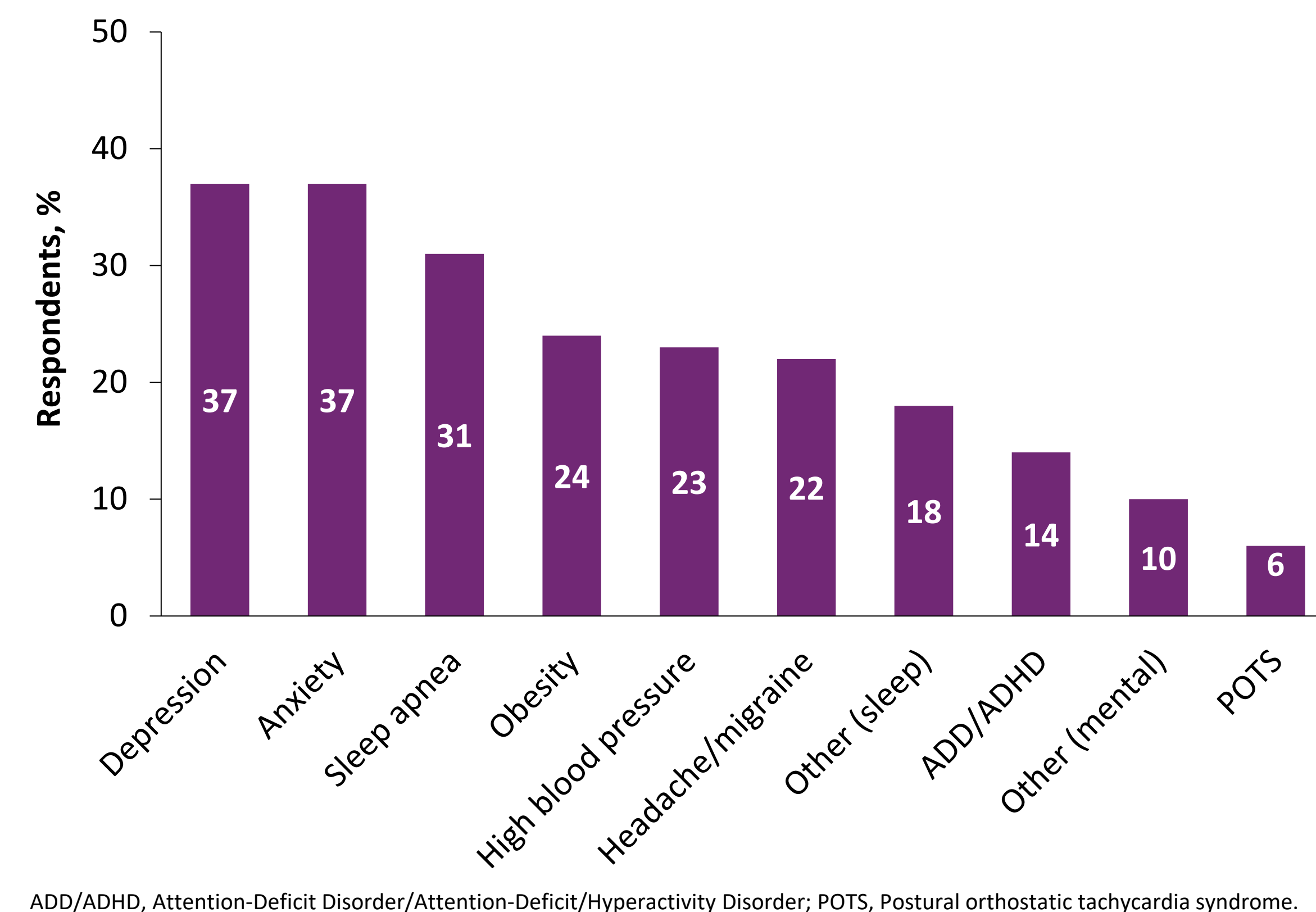
## Introduction

- Narcolepsy is a chronic, debilitating, neurological condition that dysregulates the sleep-wake cycle and is characterized clinically by excessive daytime sleepiness (EDS), cataplexy (narcolepsy type 1 [NT1] only; c. 70% of patients<sup>1</sup>), hypnagogic/hypnopompic hallucinations, sleep paralysis, and disrupted nocturnal sleep<sup>2,3</sup>
  - Narcolepsy has a substantial impact on quality of life, impairing social, professional, and physical functioning<sup>4</sup>
  - Patients with narcolepsy often require polypharmacy due the burden of the multiple symptoms experienced<sup>5</sup>
- Narcolepsy is underrecognized and underdiagnosed, partially due to symptomatic overlap with more common sleep and psychiatric conditions such as sleep apnea and depression<sup>6</sup>

## Key Findings

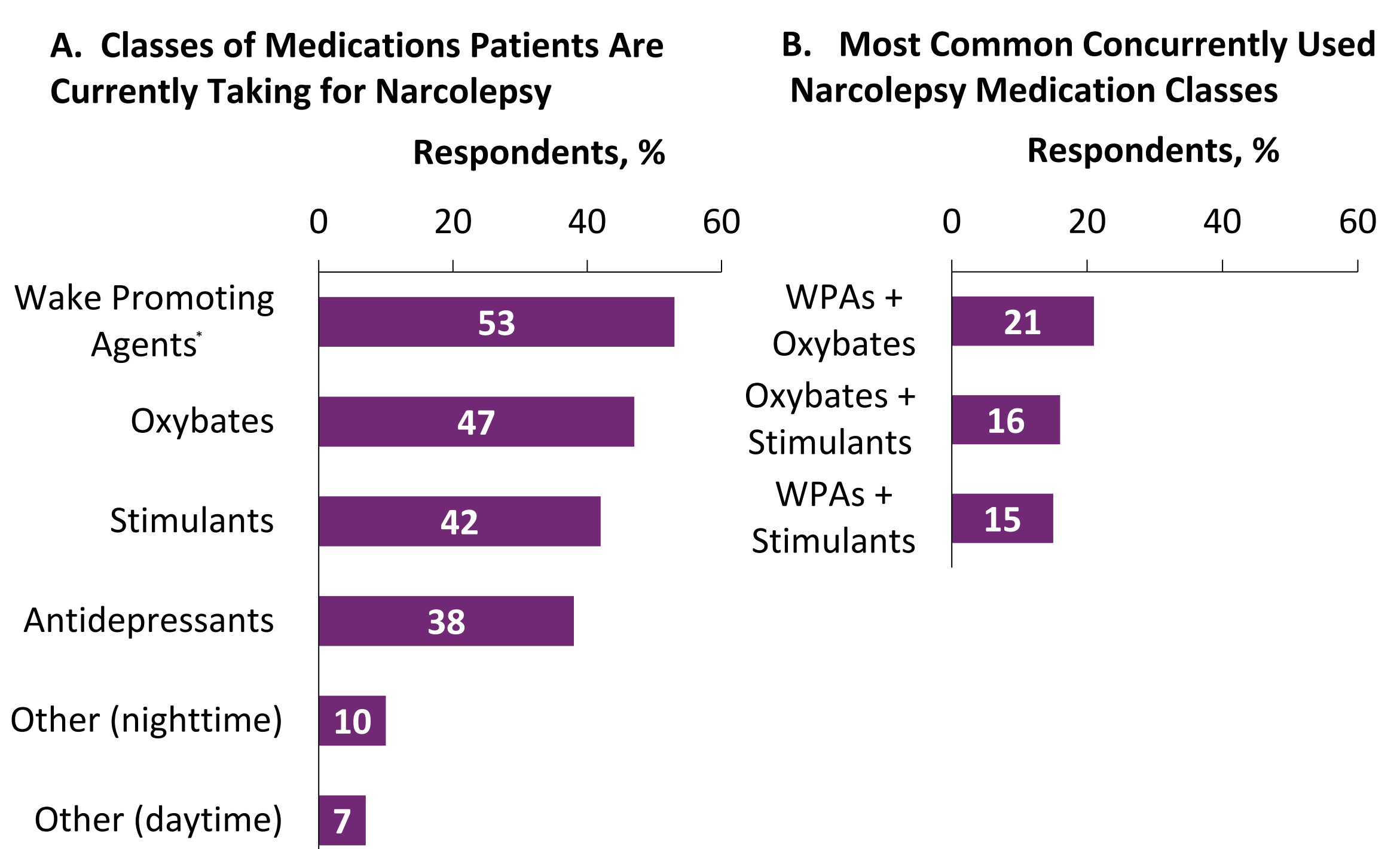
Table 1. Baseline Characteristics	Total (N=203)
Age, mean (range)	41 (18-82)
Age at symptom onset, mean (range)	18 (1-63)
Age at diagnosis, mean (range)	28 (8-67)
Gender, n (%)	
Female	130 (64)
Male	32 (16)
Transgender, Gender Variant/Non-Conforming, or Other	10 (5)
Decline to answer	31 (15)
Hispanic/Latino, n (%)	
Yes	10 (5)
No	159 (78)
Decline to answer	34 (17)
Race, n (%)	
White	143 (70)
Decline to Answer	34 (17)
Multiple	12 (6)
Black or African American	9 (4)
Other	3 (2)
Asian	2 (1)

Figure 1. Diagnosed Comorbidities Occurring in ≥5% of Participants



- Depression and anxiety were the most commonly diagnosed comorbidities reported by patients (Figure 1)

Figure 2. Medication Usage Patterns

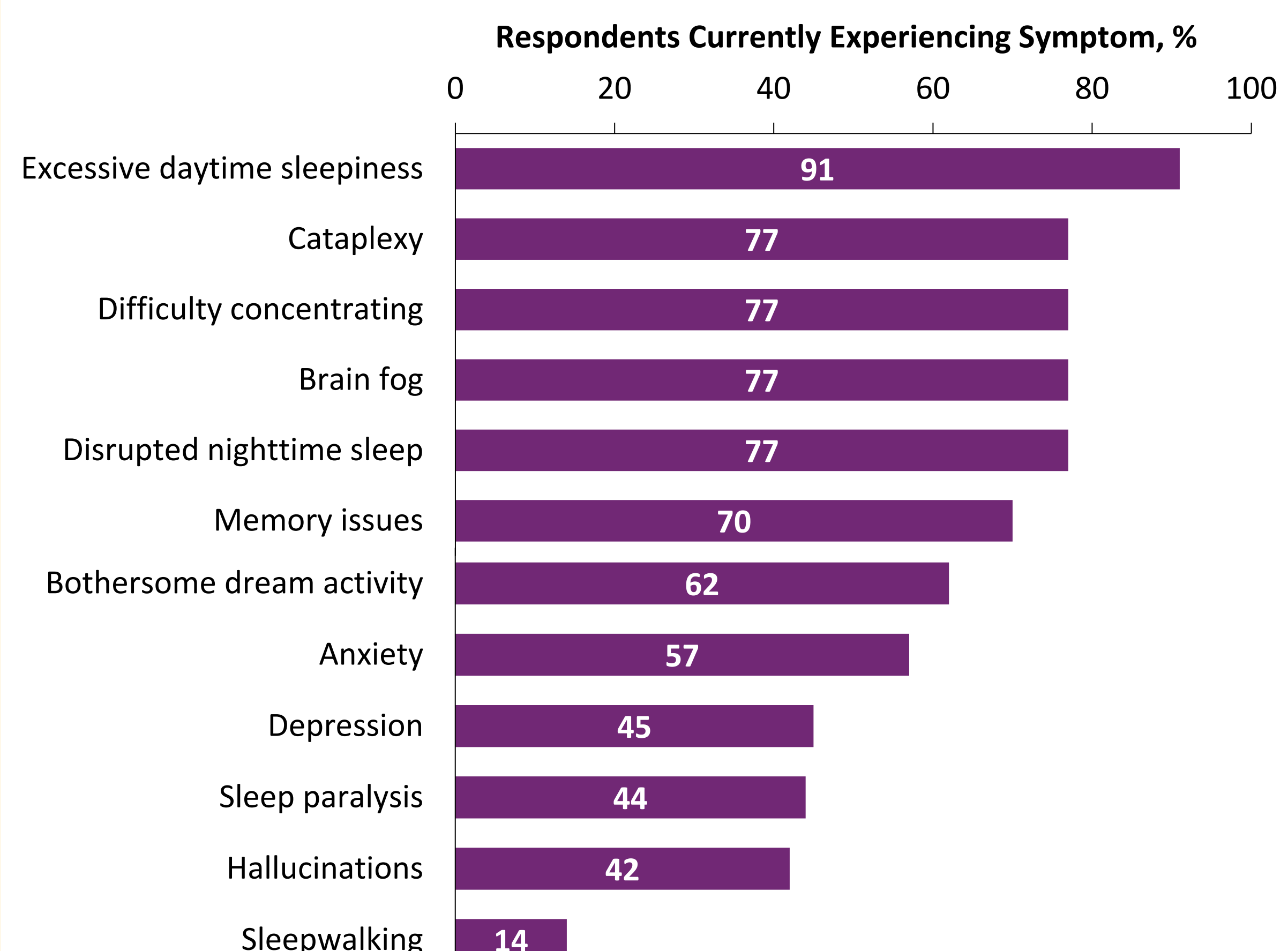


Question: Which medications do you currently take for the treatment of your narcolepsy symptoms?

\*Wake promoting agents (WPAs): armodafinil, modafinil, pitolisant, and solriamfetol.

- The most common treatments were wake-promoting agents (53%), oxybates (47%), and stimulants (42%) (Figure 2A)
- Polypharmacy for narcolepsy was common (Figure 2B; most frequently reported): overall, 30% of respondents reported taking medications from 2 classes, while 30.6% reported taking from 3 or more classes
- Almost all (93%) respondents reported discontinuing at least one medication, with lack of desired efficacy (51%), too many side effects (42%), and recommendation of new treatment by healthcare provider (34%) as the most commonly cited reasons

Figure 3. Prevalence of Breakthrough Symptoms Experienced Despite Treatment



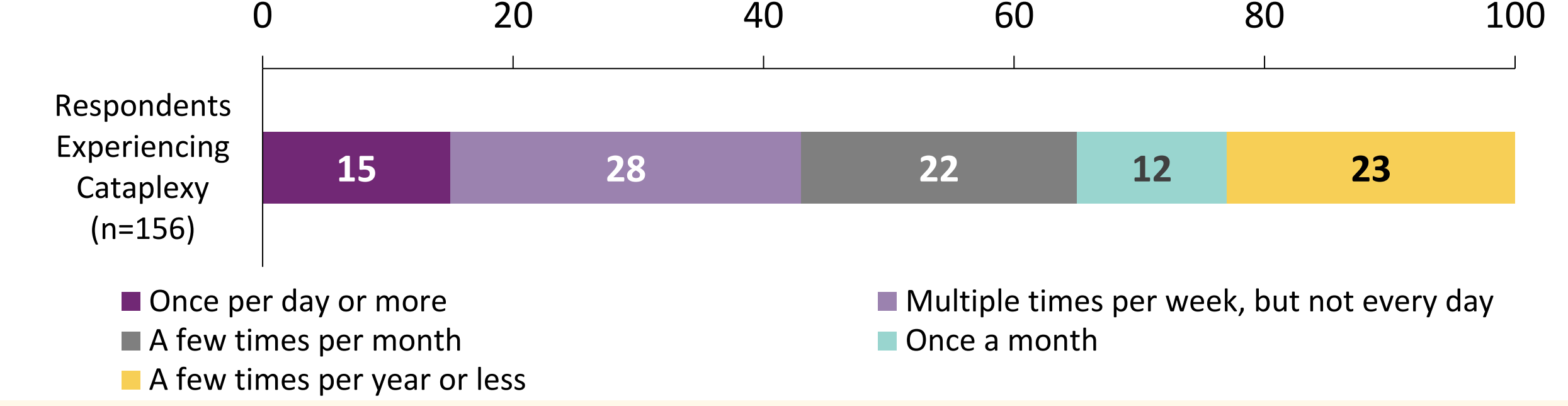
Question: What is your experience, if any, with each of the following?

- EDS was the most prevalent breakthrough symptom experienced by participants while taking their current treatment regime (91%); cataplexy, difficulty concentrating, brain fog, and disrupted nighttime sleep were the next most experienced (77% each) (Figure 3)

## Methods & Study Design

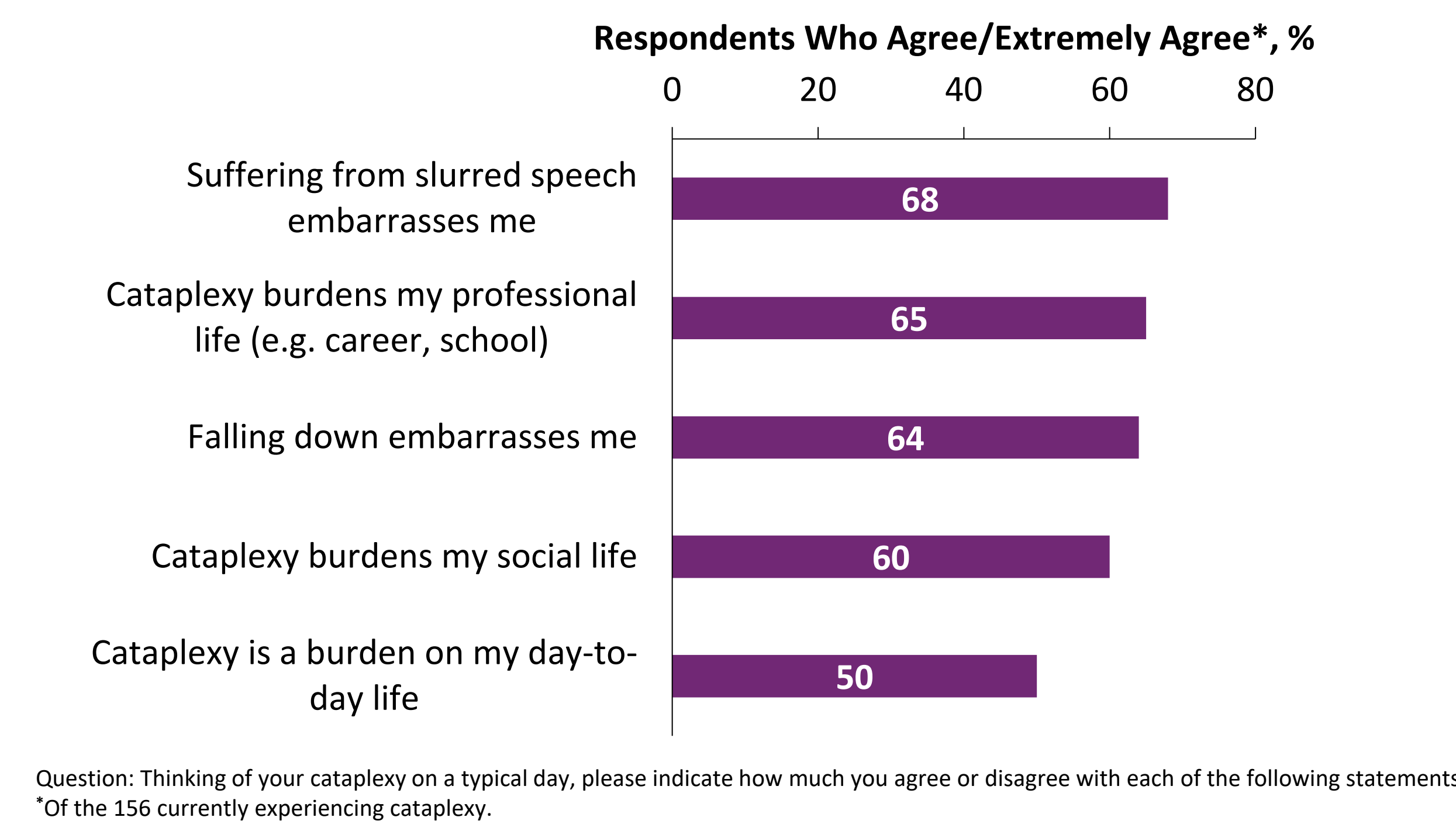
- To better understand the patient experience, burden of illness, and unmet needs for patients with narcolepsy, the Characterizing Patient Perspectives on Unmet Needs in Narcolepsy (CRESCENDO) survey was conducted from October to December 2023, involving adults diagnosed with NT1 who were currently taking US FDA-approved medication for the condition
- The survey comprehensively assessed the patient experience, including comorbidities, current medication use, symptom burden and impact on quality of life
  - Participants also completed assessments of subjective sleepiness (Epworth Sleepiness Scale; ESS), subjective cognitive function (British Columbia Cognitive Complaints Inventory; BC-CCI), and depression (Patient Health Questionnaire-8; PHQ-8)
- CRESCENDO was developed and executed in partnership with the patient advocacy organization Narcolepsy Network; a third-party research firm conducted the survey and ensured respondent privacy

Figure 4. Frequency of Cataplexy Attacks in Patients Experiencing Breakthrough Cataplexy



- Of the respondents reporting breakthrough cataplexy, 43% reported experiencing it daily or multiple times per week (Figure 4)

Figure 5. Impacts of Cataplexy on Quality of Life



Question: Thinking of your cataplexy on a typical day, please indicate how much you agree or disagree with each of the following statements? \*Of the 156 currently experiencing cataplexy.

- When asked about their typical day, a majority of patients currently experiencing cataplexy despite receiving treatment felt that this symptom caused them embarrassment and impacted their professional and social life (Figure 5)

Figure 6. Assessment of Cognitive Complaints, Sleepiness, and Depression

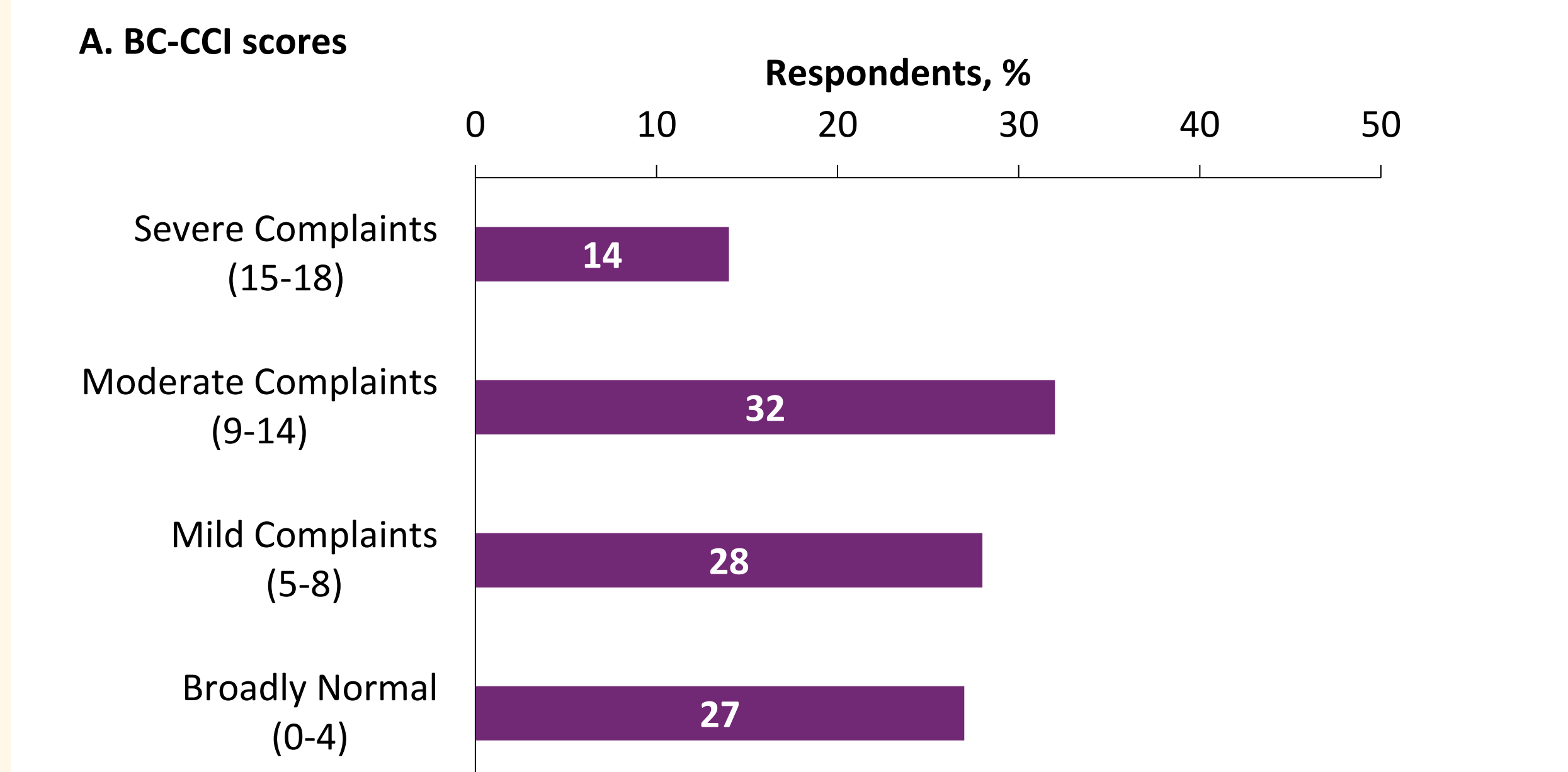
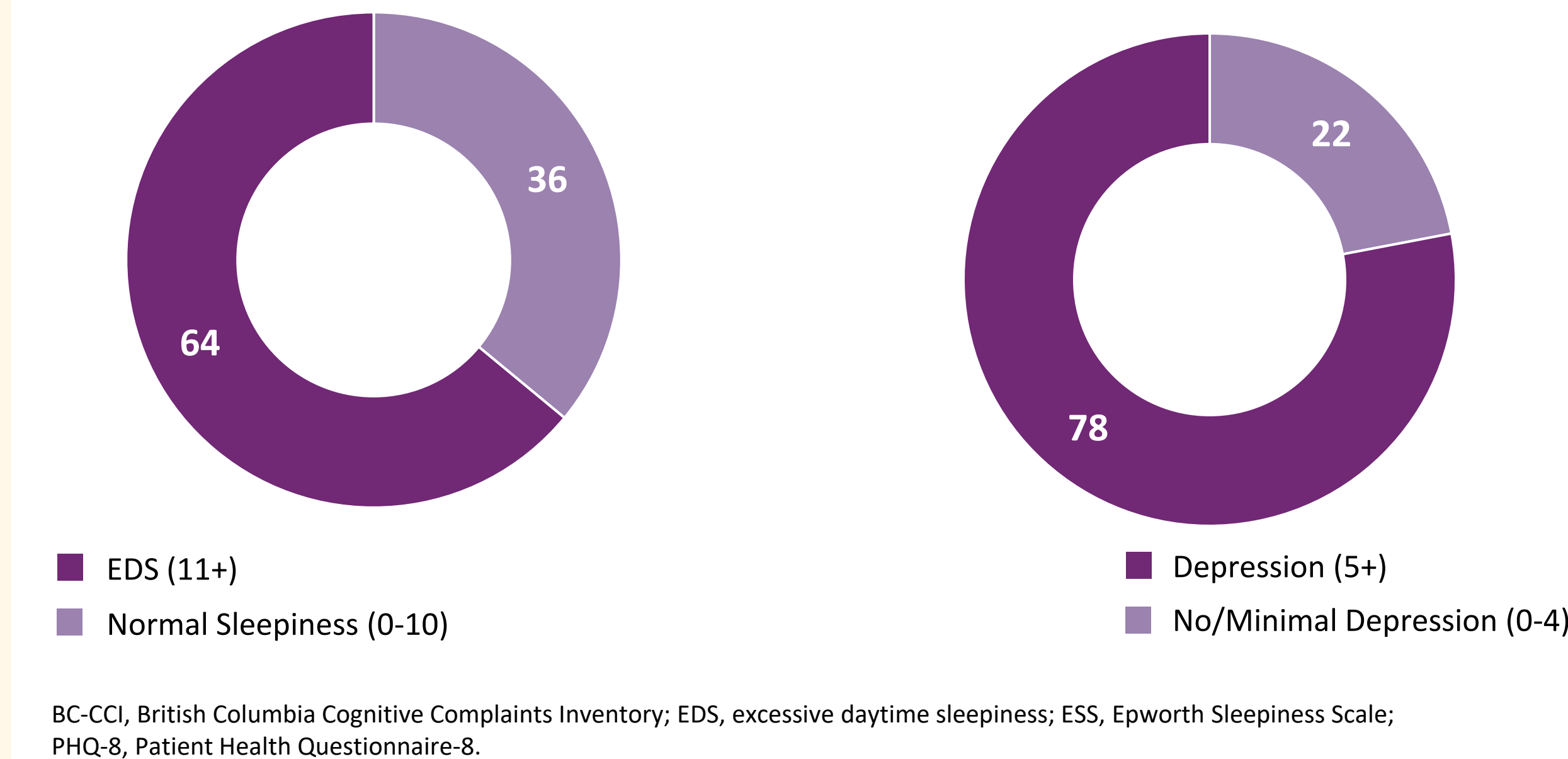


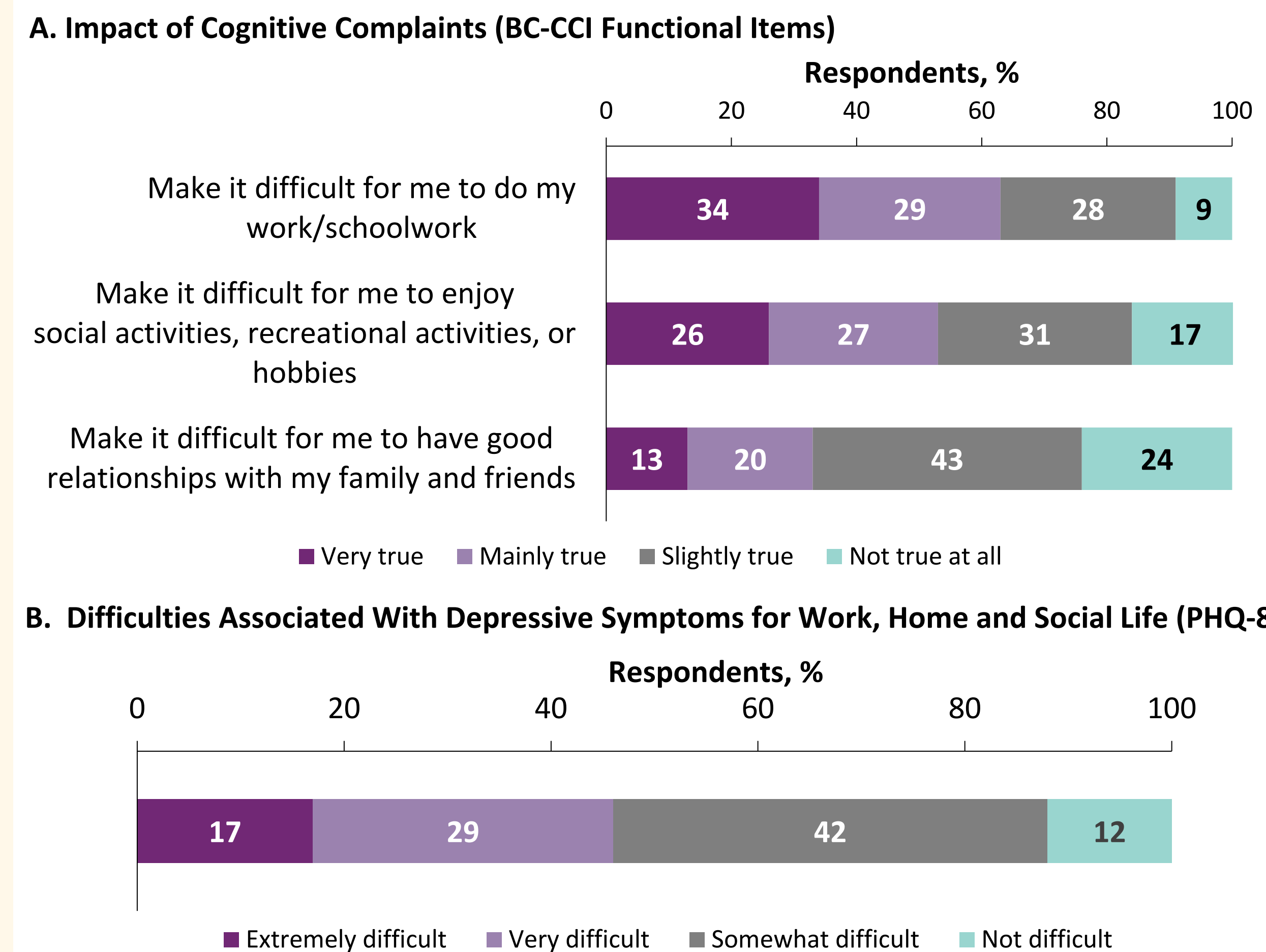
Figure 7. Quality of Life Impact of Cognitive Complaints and Depressive Symptoms



BC-CCI, British Columbia Cognitive Complaints Inventory; EDS, excessive daytime sleepiness; ESS, Epworth Sleepiness Scale; PHQ-8, Patient Health Questionnaire-8.

- Cognitive impairment (BC-CCI scores ≥5), was observed in 73% of patients (Figure 6A)
- Nearly two-thirds (64%) of participants experienced EDS, as measured by the ESS, despite receiving treatment (Figure 6B)
- More than 75% of patients presented with depression, as assessed by the PHQ-8 (Figure 6C)

Figure 7. Quality of Life Impact of Cognitive Complaints and Depressive Symptoms



Question: How difficult have these problems made it for you to do work, take care of things at home, or get along with other people? BC-CCI, British Columbia Cognitive Complaints Inventory; PHQ-8, Patient Health Questionnaire-8.

- A large majority of all respondents reported that cognitive complaints impaired their professional/scholastic lives (very/mainly/slightly true; 91%); their enjoyment of recreational pursuits (83%); and their ability to have good social relationships (76%) (Figure 7A)
- Most respondents reported that symptoms associated with depression were associated with difficulties in their professional, home, and social lives (extremely/very/somewhat difficult; 88%) (Figure 7B)