

Residual Symptom Burden in Patients With Narcolepsy Satisfied With Treatment: Subgroup Analysis From the CRESCENDO Survey

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Key Objective

- To describe the treatment experience and unmet need in patients with narcolepsy type 1 who express satisfaction with their current pharmacotherapy

Introduction

- People living with narcolepsy type 1 (narcolepsy with cataplexy [NT1]) often experience breakthrough symptoms, including excessive daytime sleepiness (EDS), cataplexy, and cognitive difficulties, even when taking multiple medications^{1,2}
- The CRESCENDO survey examined the patient experience in patients with NT1, providing a detailed characterization of symptom burden²
 - To see a previous presentation of results in the overall population, scan the QR code below:
- Here we report a subgroup analysis of respondents reporting overall satisfaction with their current pharmacologic treatment to quantify residual symptoms in this population and understand which factors differentiate these patients from those who were not satisfied

Methods

- The CRESCENDO survey was conducted from October–December 2023 in adults diagnosed with NT1 who were currently taking an FDA-approved medication for narcolepsy and included assessments of symptom burden and impact on quality of life
- 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
- Of 203 total respondents recruited, this subgroup analysis included 128 respondents who were satisfied and 75 who were not satisfied with their current narcolepsy treatment based on a 5-point categorical scale (1=very satisfied, 5=very dissatisfied)

Results

Figure 1. Treatment Satisfaction

Dissatisfied^a

Neutral

Satisfied^b

34 (17%)

41 (20%)

128 (63%)

Question: How satisfied overall are you with your current narcolepsy treatment regimen?

- Common reasons for satisfaction included symptom improvement (70%) and ability to complete more activities (37%)

Table 1. Baseline Demographic and Clinical Characteristics	Not satisfied ^a (n=75)	Satisfied ^b (n=128)
Age, mean (range)	43 (18–82)	41 (18–78)
Age at symptom onset, mean (range)	18 (5–51)	18 (1–63)
Age at diagnosis, mean (range)	30 (10–67)	27 (8–64)
Gender, n (%)		
Female	49 (65)	81 (63)
Decline to answer	9 (12)	22 (17)
Hispanic/Latino, n (%)		
No	61 (81)	98 (77)
Decline to answer	10 (13)	24 (19)
Race, n (%)		
White	56 (75)	87 (68)

References

- Swick TJ. *Nat Sci Sleep*. 2015;7:159-69.
- Thorpy MJ, et al. Presented at: American Academy of Neurology (AAN) 77th Annual Meeting; April 5–9, 2025; Copenhagen, Denmark.

Results

Figure 2. Medication Usage Patterns

A. Classes of Medications Patients Are Currently Taking for Narcolepsy

Medication Class	Not satisfied (n=75)	Satisfied (n=128)
WPA ^a	43	59
Oxybates	31	56
Stimulants	48	38
Antidepressants ^b	37	39
Other (nighttime)	8	11
Other (daytime)	4	9

B. Most Common Concurrently Used Narcolepsy Medication Classes

Medication Class	Not satisfied (n=75)	Satisfied (n=128)
WPA ^a + oxybates	11	27
Oxybates + stimulants	13	18
WPA ^a + stimulants	9	18

- A greater proportion of respondents who were satisfied with their current treatment were receiving wake-promoting agents (WPA) and oxybates than those who were not satisfied with treatment (Figure 2A)
- Polypharmacy was more common in respondents who were satisfied with treatment vs not satisfied (Figure 2B); 65.6% of satisfied respondents reported taking medications from 2 or more classes vs 52.0% of not satisfied respondents

Patients could receive more than one medication in the same class.
^aWPAs included armodafinil, modafinil, pitolisant, and solriamfetol.
^bAntidepressants for narcolepsy.
WPA, wake-promoting agent.

Figure 4. Cognitive Complaints and Impact on Quality of Life

A. Distribution of Cognitive Complaint Severity (BC-CCI)

Severity	Not satisfied (n=75)	Satisfied (n=128)
Broadly Normal (0-4)	13	35
Mild Complaints (5-8)	27	28
Moderate Complaints (9-14)	40	27
Severe Complaints (15-18)	20	10

B. Impact of Cognitive Complaints (BC-CCI Functional Items)

Not satisfied (n=75)

Item	Very true	Mainly true	Slightly true	Not true at all
Make it difficult for me to do my work/schoolwork	47	33	17	3
Make it difficult for me to enjoy social activities, recreational activities, or hobbies	43	31	17	9
Make it difficult for me to have good relationships with my family and friends	25	24	39	12

Satisfied (n=128)

Item	Very true	Mainly true	Slightly true	Not true at all
Make it difficult for me to do my work/schoolwork	27	27	34	13
Make it difficult for me to enjoy social activities, recreational activities, or hobbies	16	24	38	21
Make it difficult for me to have good relationships with my family and friends	6	18	45	31

- Respondents who were satisfied with treatment reported less severity of cognitive complaints and less of an impact on quality of life vs those who were not satisfied
- Most respondents who were satisfied with treatment still indicated at least some difficulty with work or social life due to their cognitive symptoms, indicating residual functional burden

Numbers may not add up to 100% due to rounding.
BC-CCI, British Columbia Cognitive Complaints Inventory.

Figure 6. Breakthrough Cataplexy

A. Frequency of Cataplexy Attacks in Respondents Experiencing Breakthrough Cataplexy

Frequency	Not satisfied (n=64)	Satisfied (n=92)
Once per day or more	22	11
Multiple times per week	34	23
A few times per month	23	22
Once per month	13	12
A few times per year or less	8	32

B. Impact of Cataplexy on Quality of Life

Statement	Not satisfied (n=55)	Satisfied (n=75)
Suffering from slurred speech embarrasses me	76	63
Falling down embarrasses me	69	60
Cataplexy burdens my professional life	80	53
Cataplexy burdens my social life	75	49
Cataplexy is a burden on my day-to-day life	65	39

- Respondents who were satisfied with treatment experienced attacks less often and reported less of a burden than those who were not
- Half or more of patients who were satisfied still report that cataplexy embarrasses them and burdens their work or social lives (Figure 6B)

^aPercentages based on the population experiencing breakthrough cataplexy with non-missing data.

Figure 3. Prevalence of Breakthrough Symptoms Experienced

Symptom	Not satisfied (n=75)	Satisfied (n=128)
EDS	95	89
Brain fog	84	73
Difficulty concentrating	87	72
Cataplexy	85	72
Disrupted nighttime sleep	89	70
Memory issues	80	64
Bothersome dream activity	73	56
Anxiety	69	49
Sleep paralysis	47	42
Depression	53	41
Hallucinations	59	33

- Although breakthrough symptoms were less common in respondents who were satisfied with treatment vs not satisfied, the prevalence of breakthrough symptoms remained high
- Almost all participants reported breakthrough EDS regardless of their satisfaction; however, a greater proportion of those in the not satisfied group (80%) had Epworth Sleepiness Scale scores >10 compared with those who expressed satisfaction (54%)

EDS, excessive daytime sleepiness.

Figure 5. Depressive Symptoms and Impact on Quality of Life

A. Proportion of Respondents With Depression as measured by the Patient Health Questionnaire-8 (PHQ-8)

Depression (PHQ-8 5+)	Not satisfied (n=75)	Satisfied (n=128)
Yes	96	67

B. Difficulties Associated With Depressive Symptoms for Work, Home, and Social Life (PHQ-8)

Not satisfied (n=75)

Difficulty	Extremely difficult	Very difficult	Somewhat difficult	Not difficult
How difficult have these problems made it for you to do work, take care of things at home, or get along with other people?	28	37	32	3

Satisfied (n=128)

Difficulty	Extremely difficult	Very difficult	Somewhat difficult	Not difficult
How difficult have these problems made it for you to do work, take care of things at home, or get along with other people?	10	23	48	18

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?

Extremely difficult, Very difficult, Somewhat difficult, Not difficult

- The proportion of patients with depression was higher as assessed by the PHQ-8 (Figure 5A) vs a self-report (Figure 3)
- Rates of depression were lower in respondents who were satisfied with treatment vs not satisfied, although over half of respondents who were satisfied with their treatment still reported depression
- Most respondents who were satisfied with treatment indicated at least some difficulty with work, home or social life due to depressive symptoms (Figure 5B)

Numbers may not add up to 100% due to rounding.

Conclusions

- Respondents who reported overall satisfaction with their current NT1 treatment regime experienced a lower frequency and severity of symptoms vs those who were not satisfied
- However, respondents who reported overall satisfaction with their current treatment continued to experience substantial and diverse symptoms
- These findings reveal a disconnect between patient-reported treatment satisfaction and the degree of symptom resolution, which may reflect limitations of current therapies, underreporting of ongoing symptoms, or diminished patient expectations
- Overall, the results of this study suggest the need for enhanced assessment of residual symptoms and novel approaches to treating narcolepsy

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QR Code

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Disclosures

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