

# A Qualitative Exploration of Patient and Healthcare Provider Perspectives on Oxybate Treatments for Narcolepsy

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## Introduction

- Narcolepsy is a chronic disorder characterized by dysregulation of the sleep–wake cycle. A cardinal symptom of narcolepsy is excessive daytime sleepiness.<sup>1-3</sup> High-sodium oxybate and low-sodium oxybate, both dosed twice nightly, and fixed-dose high-sodium oxybate are approved by the US Food and Drug Administration to treat excessive daytime sleepiness and cataplexy in patients with narcolepsy<sup>4-6</sup>
- Limited evidence exists on patients' experiences with treatment and healthcare provider (HCP) considerations for selecting a narcolepsy treatment
- This qualitative study examined patient and HCP perspectives regarding oxybate treatments for narcolepsy to provide insight into treatment decision-making

## Objective

- To describe patient and HCP perspectives regarding characteristics of oxybate treatments for narcolepsy

## Methods

- Qualitative, semi-structured, one-on-one telephone interviews lasting approximately 45 minutes were conducted with patients and HCPs recruited from national opt-in survey panels in December 2023–January 2024. Qualitative research experts and an internal study team employed best practices to develop an interview guide to meet the study objective of gathering insights from participants on their thoughts and experiences regarding oxybate treatments for narcolepsy
- Interview transcripts were coded using MAXQDA, a qualitative analysis software, and the codebook was refined iteratively until agreement of codes was met. Thematic analysis was performed to identify key themes
- Eligible patients were ≥18 years of age, self-reported a physician diagnosis of narcolepsy, and had been taking an oxybate treatment for ≥3 months
- HCPs were required to have been board-certified or -eligible with a specialty or subspecialty in sleep medicine, managed ≥5 patients with narcolepsy, and prescribed for ≥3 patients an oxybate treatment for narcolepsy within the past 6 months

## Results

**Table 1. Patient Characteristics**

Characteristic	Patients (N=12)
Age, years, mean (range)	46.67 (21–78)
Sex	
Female	11
Male	1
Race*	
White	10
Black or African American	2
American Indian or Alaska Native	1
Highest level of education completed	
High school graduate or equivalent (eg, GED diploma)	0
Associate degree or technical school graduate	3
Completed some college or technical school, but no degree	2
College graduate (eg, AB, BA, BS degrees)	3
Completed graduate school (eg, MD, MS, PharmD, PhD degrees)	4
Narcolepsy type	
Type 1	3
Type 2	9
Current narcolepsy treatment(s) <sup>†</sup>	
High-sodium oxybate only	2
Low-sodium oxybate only	3
High-sodium oxybate plus stimulant(s)	3
Low-sodium oxybate plus stimulant(s)	4

\*One participant reported being White and American Indian or Alaska Native.

<sup>†</sup>None of the patients taking oxybate treatments for narcolepsy were taking fixed-dose high-sodium oxybate. AB, bachelor of arts; BA, bachelor of arts; BS, bachelor of science; GED, General Educational Development; MD, doctor of medicine; MS, master of science; PharmD, doctor of pharmacy; PhD, doctor of philosophy.

**Table 2. Healthcare Provider Characteristics**

Characteristic	Healthcare Providers (N=10)
Type of specialist, n	
Neurologist	6
Pulmonologist	2
Primary care physician	1
Internist	1
Years in practice, mean (range)	20.6 (4–30)
Number of patients with suspected or diagnosed narcolepsy actively managed within past 6 months, mean	65.2
Number of patients with narcolepsy prescribed oxybate treatment within past 6 months, mean	29

**Figure 1. Patient Perceptions of Benefits of Oxybate Treatment for Narcolepsy**

*"It was a total game changer for the cataplexy. In the morning, I actually wake up and feel like I've actually slept for the first time since I developed narcolepsy. That helps with having more energy and focus throughout the day than I did when I was just on stimulants."* — Patient 03

### Patient Perceptions of Benefits

*"[My oxybate treatment] has definitely helped me in the sense of staying asleep and making sure I do stay asleep. Also, it kind of helps with the dreams too. I do feel a lot more relaxed with it."* — Patient 06

- Overall, most patients reported that their oxybate treatment has had a positive impact on their lives, as they can engage in daily activities, such as having conversations and watching TV or movies, without falling asleep, and they can concentrate while at work or school

**Figure 2. Efficacy/Effectiveness Is the Most Important Outcome for Both Patients and HCPs**

*"Once I came to the realization how much I was sleeping, and how much quality of life I was losing out on, cutting down on the napping throughout the day was the biggest part for me."* — Patient 05

*"The choice, of course, is really you focus on two things. Like all other medical conditions. One is the efficacy, the benefits. Second, of course, is the adverse effects. Any risks. Any medical condition, you have to balance these two."* — HCP 01

### Efficacy

*"I liked taking a medication that helped me get the restful sleep versus taking a stimulant in the daytime to stay awake. . . . I definitely like . . . that good quality night's sleep."* — Patient 07

*"The number one factor is the efficacy, that it works. For any drug that I use, that's by far and away the number one driving factor."* — HCP 03

**Figure 3. Sodium Content Is a Treatment Consideration for Both Patients and HCPs**

*"I looked into it because I was interested but it's back up to a significant amount of sodium. I would rather take two doses of a lower sodium versus one high sodium medication."* — Patient 07



*"[Adding] more salt on people doesn't make sense. . . . You have a chance to keep people healthy. I see no reason why not."* — HCP 03

- All HCPs in this study considered sodium content when prescribing oxybate treatments; some preferred prescribing a low-sodium oxybate to patients with cardiovascular disease or other underlying health risks for whom excess sodium is a concern
- Many patients also noted sodium content as important, given the potential long-term health consequences, such as hypertension
- Some patients who were not concerned about sodium content explained that they were otherwise healthy, but it would be important to monitor their blood pressure and cholesterol to ensure there were no issues

**Figure 4. Importance of Individualized Dosing**

### Titration With Precision

*"When I was titrating up [I changed dosing], but once I got to my therapeutic dose I've never gone back."* — Patient 07



*"I think having the ability to individualize dosing is very important because every patient is different. I think we need to have it, in order to minimize the risk of side effects, and to get the optimal dose."* — HCP 02

### Symmetric/Asymmetric Dosing

*"To me, it allows me to have some freedom . . . Knowing that I have that bit of control that if, when I need to, I can adjust the dosage [under my doctor's guidance]."* — Patient 06



*"I have patients [who] have an uneven dosing. . . . [It's] pertinent to some patients . . . For instance, maybe a patient likes to do a higher dose at bedtime if they have a hard time falling asleep."* — HCP 07

### Tailor Timing of Second Dose (per physician guidance)

*"You can take [the second dose] two and a half hours to four hours after your first dose. [I do that now.] . . . I like that you have that ability to do that if you wake up before your next dose and it's already been enough time, you can go ahead and take your medication and go back to sleep."* — Patient 02



*"It's important to have that assurance that [the timing of the dose] can be variable, that there's not . . . strict timing. Like on the fifth hour you need to do this, it has to be exactly on the fifth hour, it's not that way."* — HCP 06

- Individualized dosing is a key treatment attribute, as it allows patients, per physician guidance, to tailor the dose to their needs and reduce or prevent side effects
- Many patients reported titrating to reach an optimal dose and then adjusting their dose to accommodate their sleep schedule or need to travel the next morning—a sentiment echoed by HCPs who described the value of individualized dosing of treatment for patients with narcolepsy

**Figure 5. Participant Dosing Frequency Considerations**

*"If I took it once nightly and I could sleep through the night, then I wouldn't have a break in my sleep."* — Patient 08

### Dosing Frequency

*"It's not a godsend, new thing that I have to offer patients. When a new drug comes out sometimes you get excited, but I can't say that I'm excited about [the once-nightly]. Because it doesn't offer me anything much more than what I already have."* — HCP 03

*"The one concerning thing [about once-nightly] would just be . . . once you take it, you're in it for a bigger chunk of time. In terms of how long am I going to be in a deep sleep, would it impact me differently in terms of my ability to be awake and alert and responsive in case of an emergency."* — Patient 11

*"If a patient comes up to me and says, 'Okay, I'm really struggling taking these medications twice a night because I have kids and I have this and it's hard for me to fall back asleep.' This is a patient where I would recommend doing the one nightly regimen."* — HCP 08

- A few HCPs found that the benefit of the once-nightly treatment was offset by its high sodium content
- The patients who preferred twice-nightly administration felt:
  - Less concerned about falling into a deep sleep that might make it difficult to wake up or be responsive in an emergency
  - Less scared about taking a short-acting medication, as their perception was that it would have fewer side effects
  - Twice-nightly administration worked best for their body's metabolism
- HCPs shared that twice-nightly administration may be difficult for patients who have busy schedules, as they may feel groggy after taking the second nighttime dose

## Conclusions

- In this exploratory qualitative study, symptom relief emerged as the leading treatment consideration for patients; sodium content and dosing flexibility were among other characteristics considered
- Effective symptom relief was also a consideration for HCPs selecting an oxybate treatment for narcolepsy, followed by sodium content
  - Patient safety was also an important consideration
- Patients and HCPs valued the ability to adjust dosing, per physician guidance, according to patient needs and response
- The study's data were collected from a relatively small number of individuals, and thus it is possible that new themes might have been identified in additional interviews with other respondents. Further research with a large, diverse sample may assist in enhancing knowledge on this topic

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