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

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Background

- Narcolepsy is a chronic disorder characterized by the dysregulation of the sleep/wake cycle; a cardinal symptom is excessive daytime sleepiness (EDS).[1,2]
- High-sodium oxybate and low-sodium oxybate, both dosed twice nightly, and fixed-dose high-sodium oxybate are approved to treat EDS and cataplexy in patients with narcolepsy.[3,4,5]
- Limited qualitative literature exists on patient and healthcare healthcare provider's (HCP) perspectives on the treatment experience with oxybates for narcolepsy. This qualitative study examines patient and HCP perspectives regarding characteristics of 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

- One-on-one, semi-structured, qualitative telephone interviews lasting approximately 45 minutes were conducted with patients and HCPs recruited from national opt-in survey panels.
- Interview transcripts were coded using MAXQDA, a qualitative analysis software, and the codebook was refined using an iterative approach until agreement of codes were met. Thematic analysis was conducted to identify key themes.
- Eligible patients were aged ≥18 years who self-reported a physician diagnosis of narcolepsy and have been taking an oxybate treatment for ≥3 months.
- HCPs must have been board-certified or board-eligible with a specialty/subspecialty in sleep medicine; managed ≥5 patients with narcolepsy and prescribed ≥3 patients an oxybate treatment for narcolepsy in the past 6 months.

Results

- Eight patients and seven HCPs were interviewed in December 2023.
- Participant demographics are presented in **Table 1** and **Table 2.**
- Patients reported that their primary current narcolepsy symptom was EDS; other symptoms included poor sleep quality, fatigue, feeling "groggy," experiencing hallucinations, as well as cataplexy attacks for those with type 1 narcolepsy.
- Currently, patients with narcolepsy have managed their symptoms with oxybate treatments, daytime stimulants, and lifestyle changes such as eating healthy, exercising, practicing good sleep hygiene, and taking naps.

Table 1. Patient Demographics

Variable	
Age	Mean 47.5 ± 16.8
Gender	
Female	7
Male	1
Race[a]	
White	6
Black or African American	2
Ethnicity	
Non-Hispanic	7
Hispanic	1
Highest Level of Education Completed	
High school graduate or equivalent (e.g., GED)	0
Associate degree or Technical school graduate	3
Completed some college or technical school, but no degree	2
College graduate (e.g., BA, AB, BS)	1
Completed graduate school (e.g., MS, MD, PhD, PharmD)	2
Narcolepsy Type	
Type 1	2
Type 2	6
Current Narcolepsy Treatment(s)	
High-sodium oxybate only	1
Low-sodium oxybate only	2
High-sodium oxybate plus a stimulant(s)	2
Low-sodium oxybate plus a stimulant(s)	3

[a]One participant reported being White and American Indian or Alaska Native

Table 2. HCP Demographics

Variable	
Profession	
Neurologist	4
Pulmonologist	2
Primary Care Physician	1
	Means
Years in Practice	23.9 ± 4.4
Patients Actively Managed in P6M* with Suspected or Diagnosed Narcolepsy	69.6 patients
Patients with Narcolepsy Prescribed Prescribed Oxybate Treatment in P6M*	29.3 patients

*P6M: past 6 months

Treatment Experiences

- Many patients noted that their oxybate treatment effectively managed their narcolepsy symptoms, including daytime sleepiness, and helped them fall asleep, stay asleep, and wake up feeling rested.
- 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.
- Importantly for their quality of life, treatment has translated into patients not having the urge/need to nap throughout the day as often, therefore not being in bed all the time, and having energy throughout the day.
- Patients shared that when starting an oxybate treatment, their dose was adjusted until side effects were minimized and it effectively addressed their symptoms. Adhering to the treatment instructions, including taking medication at least 2 hours after eating, eventually became routine.

Oxybate Treatment Considerations

- Treatment effectiveness was the primary consideration when selecting an oxybate treatment for both patients and HCPs.
- Sodium content, dosing frequency and individualized dosing were also considered important by many participants.
- Given the low potential risk of side effects and the ability to address them with titration, patients and HCPs were not as concerned with side effects when considering a new treatment.
- Considering safety risks, including central nervous system depression, abuse and misuse, HCPs may also consider alternatives to available oxybate treatments for those with a history of, or current substance use disorder, those taking opioids, and those with mental health conditions such as depression.

Efficacy

- Among the key treatment characteristics reported by HCPs and patients, symptom relief emerged as most important, as a reduction in symptoms may mean an improvement in quality of life.

Patients

"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." **PT05**

"I liked taking a medication that helped me get the restful sleep versus taking a stimulant in the daytime to stay awake. I like that I can get that good quality night's sleep." **PT07**

"The helping with my cataplexy and the sleep inertia, those were really important to me for reasons for taking it. Hopefully to get back to where I'm able to go back to work at some point." **PT02**

HCPs

"Of course, it is to try to prevent daytime sleepiness. Sometimes you try to prevent cataplexy attacks. That's the goal. There's no cure, of course, at this moment. We try to give them their quality of life back." **HCP01**

"By far and away, the most common problem they struggle with is daytime sleepiness which affects their job, their schooling, all they have to do. Everything that we do is to improve that daytime sleepiness, so they can function, maybe not normal, but as close to normal as possible." **HCP03**

"My goal really and always is to try to heed the patient's requests what's going to afford them the best quality of life. Oftentimes my goal is to have a patient on a regimen that gives them good quality of life, minimizes any potential adverse things that could happen as a result of the sleepiness in the daytime, whether it's work related or anything in their personal life." **HCP04**

Sodium Content

- All HCPs in this study consider sodium content when prescribing oxybate treatments.
- Some preferred prescribing a low-sodium oxybate to patients with cardiovascular disease or other underlying health risks where excess sodium is a concern.
- Many patients also noted sodium content as important, given potential long-term health consequences such as hypertension.

Patients

"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." **PT07**

"My doctor said, "Your cardiologist would be happy." I have hypertension, which is well-managed. That's the main reason we switched [to low sodium oxybate]." **PT01**

HCPs

"I do prefer the low sodium oxybate treatment better because of the sodium load." **HCP05**

"Adding more salt on people doesn't make sense. You have a chance to keep people healthy. I don't see a reason why not." **HCP03**

"When you take this high-sodium content, you have to really watch the diet and other sources of sodium there. You have to restrict it, but if you use a low sodium oxybate, I don't think you need to worry too much." **HCP01**

Frequency of Administration

- Frequency of administration was less important as patients were accustomed to the routine of taking twice-nightly treatment, a sentiment also shared by HCPs.
- When selecting a treatment, some patients shared that high-sodium content would be more influential compared to frequency of treatment administration.
- However, HCPs noted that twice-nightly administration may be difficult for patients who have busy schedules, particularly in the morning.

Patients

"That is not a huge factor for me. With taking a [low sodium oxybate] once before bed and once in the middle of the night, I'm so used to it now. Even in the beginning, it wasn't really a concern for me." **PT05**

"How often I need to take the medication doesn't really factor into my decision either. I make it work with the two that I take now. I don't know, it doesn't really factor into it." **PT02**

Individualized Dosing

- Individualized dosing was another key treatment attribute, as it allows patients, under their doctor's guidance, to tailor the dose to their needs and reduce or prevent side effects **(Table 3)**.

Table 3 – Individualized Dosing

	Patients	HCPs
Titration with precision		
	Many reported titrating to reach an optimal dose, under their doctor's guidance. Others who have not adjusted their dose after titration felt it would be a helpful feature that would allow for flexibility in their life.	Most described the importance of being able to titrate with precision and HCPs note that every patient is different and a dose that works for one individual may not work for all patients.
Symmetric/asymmetric dosing		
	Some described adjusting the amount of medicine in their second dose to avoid drowsiness the next morning.	HCPs reported adjusting patients' second dose to accommodate their schedule, while noting it is important to make it simple for patients.
Tailor timing of second dose (per physician guidance)		
	Patients mentioned changing when they take their second dose to accommodate their sleep schedule or in an effort to avoid drowsiness the next morning.	Having flexibility on when the second dose of an oxybate can be taken is valued by HCPs, with several commenting they often offer this option to their patients.

HCPs

"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. If there's only one drug and one dose it doesn't sound like there's a lot of potential optimization for that." **HCP02**

"Being able to individualize dosing is nice because of the side effects and because of people's routine. … Obviously, if it was a pill, if it was a fixed dose, it would be very difficult to adjust most of the time. I might use it a lot less frequently, so being able to do that is a big help." **HCP03**

Conclusions

- This exploratory qualitative study examined the characteristics considered in oxybate treatment selection for narcolepsy to better understand treatment decision-making for both patients and HCPs.
- HCPs expressed that effective symptom relief was the leading consideration when selecting an oxybate treatment for narcolepsy, followed by sodium content.
  – Safety risks (e.g., CNS depression, misuse, abuse) were also important considerations for HCPs when considering an oxybate treatment for their patients.
- Symptom relief also emerged as an important oxybate treatment characteristic for patients; dosing frequency and sodium content were among other key considerations.
  – Frequency of administration and individualized dosing under a doctor's guidance was helpful to tailor the dose to their needs and reduce medication side effects.
- Findings from this study highlight the multifaceted considerations (e.g., oxybate formulation characteristics, flexibility, and dosing frequency) that patients and HCPs take into account when selecting an oxybate treatment for narcolepsy.
- The data was collected from a relatively small number of individuals, and thus it is possible that new themes may have been identified if additional respondents were interviewed. Further research with a large, diverse sample may assist in enhancing knowledge of this topic.

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