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Real-World Survey of Treatment Effectiveness and Satisfaction in Adults With Narcolepsy Taking Low-Sodium Oxybate

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Introduction

- Low-sodium oxybate (LXB; Xywav®) is approved by the US Food and Drug Administration to treat idiopathic hypersomnia in adults and excessive daytime sleepiness (EDS) or cataplexy in patients aged ≥7 years with narcolepsy[1-4]
- Limited evidence exists on the real-world patient experience of individuals with narcolepsy taking LXB
- The **C**linical effectiveness, treatment ad**H**erence, and treatment satisfaction in adults with **I**diopathic hypersomnia and narcolepsy taking low-sodiu**M** oxybat**E** (CHIME) study evaluated real-world patient-reported outcomes, including clinical effectiveness, treatment adherence, and treatment satisfaction among adults with narcolepsy or idiopathic hypersomnia taking LXB
  - Results for individuals with idiopathic hypersomnia are reported separately in **Poster 534**

Objective

- To evaluate real-world patient-reported outcomes, including treatment effectiveness, adherence, and satisfaction among adults with narcolepsy taking LXB

Methods

- A cross-sectional, web-based survey was administered to US adults taking LXB from 08/26/2024 to 12/12/2024
  - Participants had previously consented to outreach from the study sponsor with opportunities to participate in research
- Key inclusion criteria
  - US residents ≥18 years of age with a physician diagnosis of narcolepsy (type 1 [NT1] or type 2)
  - Currently taking LXB for treatment of narcolepsy
  - Opted-in to receiving marketing/promotional communications from the study sponsor
- Key exclusion criteria
  - Current diagnoses of both narcolepsy and idiopathic hypersomnia
  - Cognitive difficulties or impairment that would make completing the survey challenging or prevent from completing the survey accurately
- Descriptive analyses were conducted on standardized patient-reported outcome measures (including the Epworth Sleepiness Scale [ESS], score range 0–24, and Patient Global Impression of Change [PGI-C]) and de novo questions to evaluate the experiences of individuals with narcolepsy taking LXB

Results

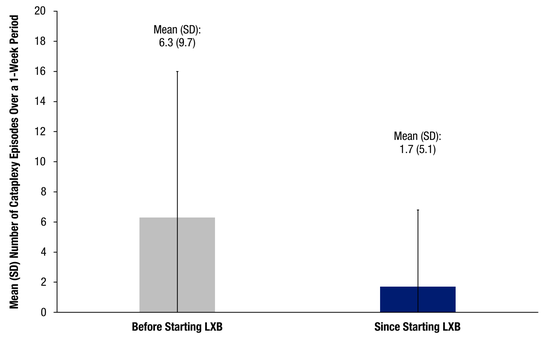
Table 1. Self-Reported Demographics and Clinical Characteristics

Characteristic	Participants With Narcolepsy (N=217)
Narcolepsy type, n (%)	
Type 1	94 (43.3)
Type 2	123 (56.7)
Age (years)	
Mean (SD)	39.6 (12.6)
Median (min, max)	37.0 (18.0, 91.0)
Gender identity, n (%)	
Female	158 (72.8)
Male	49 (22.6)
Nonbinary	4 (1.8)
Transgender male	3 (1.4)
Gender nonconforming	2 (0.9)
Prefer not to answer	1 (0.5)
Other	0
Hispanic or Latino, n (%)	
Yes	17 (7.8)
No	200 (92.2)
Race, n (%)	
White	192 (88.5)
Multiple selected	14 (6.5)
Asian or Asian American	7 (3.2)
Black or African American	4 (1.8)
American Indian or Alaska Native	0
Native Hawaiian or other Pacific Islander	0
Prefer not to answer	0
Highest level of education, n (%)	
High school graduate or less	9 (4.1)
Associate's degree or some college (no degree)	51 (23.5)
Bachelor's degree	94 (43.3)
Master's degree or doctoral/doctorate degree	61 (28.1)
Other	2 (0.9)
Number of comorbidities, n (%)	
≥1	193 (88.9)
≥2	170 (78.3)
Most common comorbidities (≥15%), n (%)	
Anxiety	124 (57.1)
Depression	101 (46.5)
Migraine/headaches	52 (24.0)
Hypertension	50 (23.0)
ADD/ADHD	45 (20.7)
Obstructive sleep apnea	45 (20.7)
Overweight	38 (17.5)
Other	34 (15.7)
ESS total score[a]	
Mean (SD)	8.3 (4.9)
Median (min, max)	8.0 (0.0, 22.0)
ESS total score by severity,[a] n (%)	
Normal (0–10)	148 (68.5)
Mild (11–12)	24 (11.1)
Moderate (13–15)	23 (10.6)
Severe (16–24)	21 (9.7)
Time taking LXB, weeks	
Mean (SD)	147.1 (57.2)
Total nightly dosage of LXB, g, n (%)	
<2.5	1 (0.5)
2.5 to 4	3 (1.4)
>4 to 6	25 (11.5)
>6 to 9	185 (85.3)
>9	3 (1.4)
Taking ≥1 concomitant alerting agent,[b] n (%)	147 (67.7)

[a]A single item had one missing value. [b]Alerting agents are defined as wakefulness-promoting agents (ie, armodafinil, modafinil, pitolisant, solriamfetol) or traditional stimulants (ie, amphetamines, methylphenidate).
ADD, attention-deficit disorder; ADHD, attention-deficit hyperactivity disorder; ESS, Epworth Sleepiness Scale; LXB, low-sodium oxybate; max, maximum; min, minimum; SD, standard deviation.

- Among the 217 participants with narcolepsy, the mean (standard deviation [SD]) time taking LXB was 147.1 (57.2) weeks, or 2.8 (1.1) years, with 74.2% taking LXB for >2 years
- Most participants (72.8%) were female and White (88.5%); mean (SD) age was 39.6 (12.6) years; 43.3% of participants had NT1

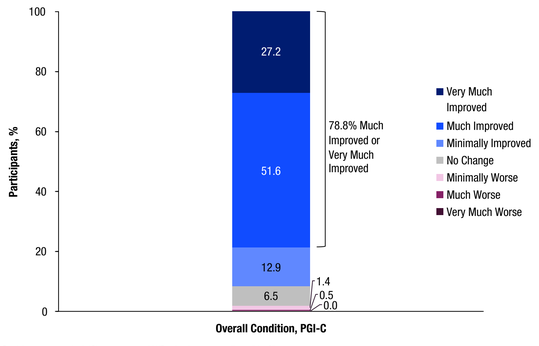
Figure 1. Mean Number of Cataplexy Episodes Over a 1-Week Period[a,b]



[a]Narcolepsy type 1 only. [b]Participants were asked, "Before you started taking LXB, how many cataplexy episodes (episodes where you lost partial or complete muscle tone while you were awake), on average, would you experience in a 1-week period?" and "Since you started taking LXB, how many cataplexy episodes did you experience in the past week?"
LXB, low-sodium oxybate; SD, standard deviation.

- Participants with NT1 (n=94) taking LXB reported fewer cataplexy episodes over a 1-week period since starting LXB

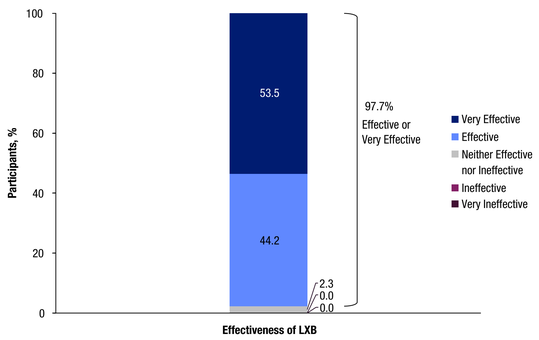
Figure 2. Patient Global Impression of Change in Overall Condition Since Starting LXB[a]



[a]Participants were asked, "Since you started LXB, what is your overall condition?"
LXB, low-sodium oxybate; PGI-C, Patient Global Impression of Change.

- On the PGI-C, 78.8% of participants reported their overall condition was "much improved" or "very much improved" since starting LXB

Figure 3. Patient-Reported Treatment Effectiveness of LXB[a]



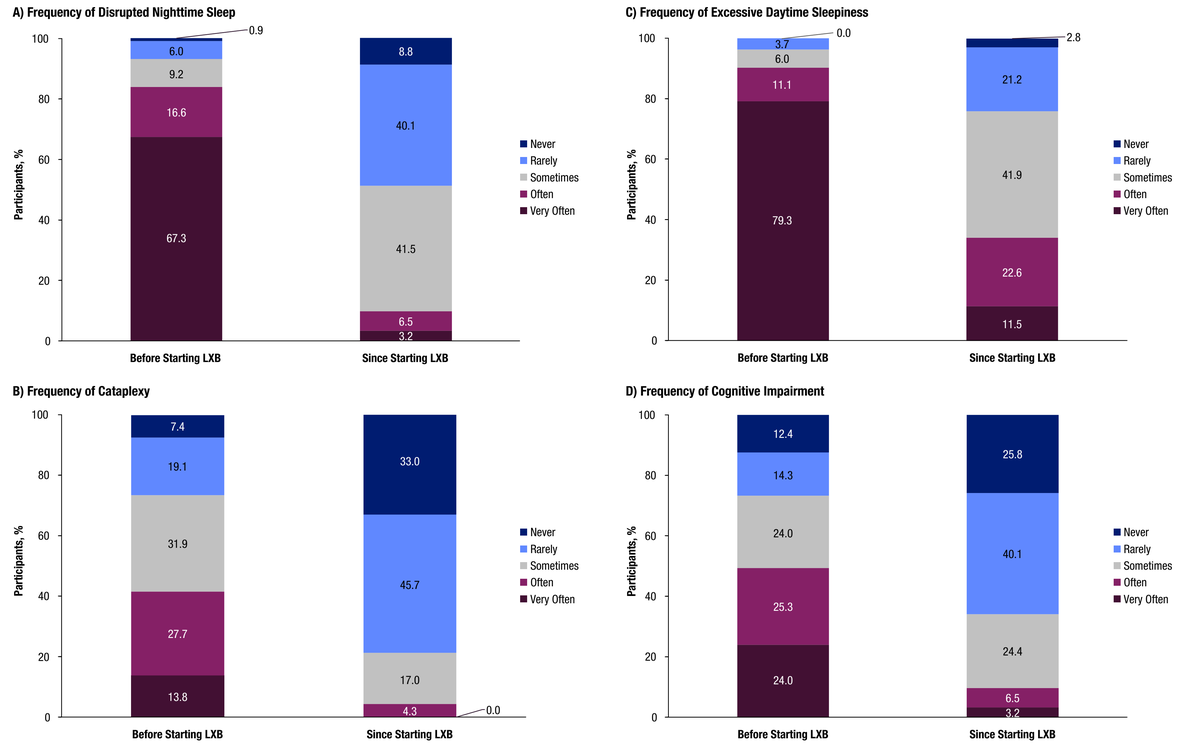
[a]Participants were asked, "Overall, how effective is LXB at managing your symptoms?"
LXB, low-sodium oxybate.

- Most participants (97.7%) reported LXB was "effective" or "very effective" at managing their narcolepsy symptoms
- Since starting LXB, 41.0% of participants stopped taking, reduced the dosage, or reduced the frequency of taking alerting agents for narcolepsy (a wakefulness-promoting agent or a traditional stimulant)

Conclusions

- This analysis from CHIME, the largest real-world survey of people taking LXB to date, suggests that participants experienced improvement in multiple symptoms of narcolepsy (including EDS, cataplexy, DNS, and cognitive impairment); and many stopped taking, reduced the dosage, or reduced the frequency of taking alerting agents for narcolepsy after starting LXB
- According to the CHIME survey findings, the ability to individualize dosing with LXB was highly important, and many participants were satisfied with the ease of taking LXB
- Limitations of this analysis include the cross-sectional design and the potential for selection bias limiting generalizability, as participants who are satisfied with LXB may have been more likely to enroll in the study

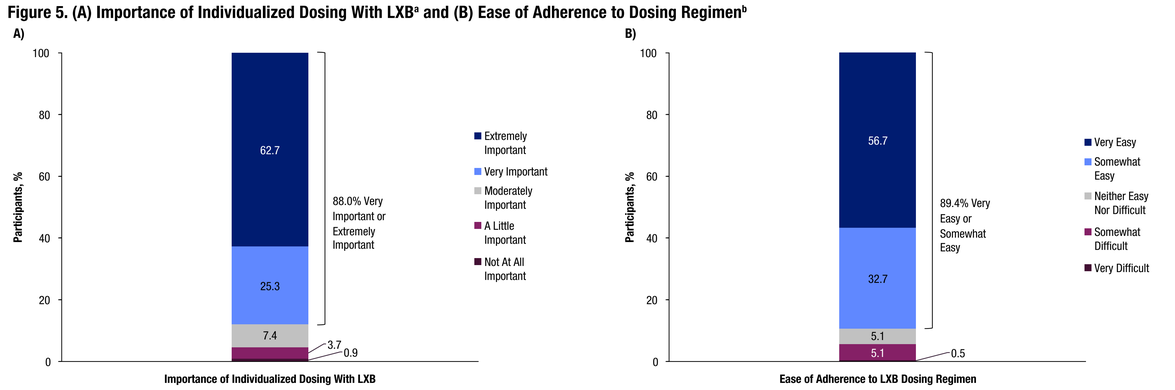
Figure 4. Patient-Reported Frequency of Narcolepsy Symptoms Before and Since Starting LXB: (A) Disrupted Nighttime Sleep;[a] (B) Cataplexy;[b,c] (C) Excessive Daytime Sleepiness;[d] and (D) Cognitive Impairment[e]



[a]Participants were asked, "How frequently did/do you experience disrupted nighttime sleep?" [b]Sample is those who indicated they previously had a diagnosis of narcolepsy type 1 (n = 94). [c]Participants were asked, "How frequently did/do you experience sudden partial or complete loss of muscle tone when awake (cataplexy)?" [d]Participants were asked, "How frequently did/do you experience excessive daytime sleepiness?" [e]Participants were asked, "How frequently did/do you experience cognitive impairment?"
LXB, low-sodium oxybate.

- Since starting LXB, the percentage of participants who reported experiencing narcolepsy symptoms "often" or "very often" decreased from 83.9% to 9.7% for disrupted nighttime sleep (DNS), 41.5% to 4.3% for cataplexy (NT1), 90.3% to 34.1% for EDS, and 49.3% to 9.7% for cognitive impairment

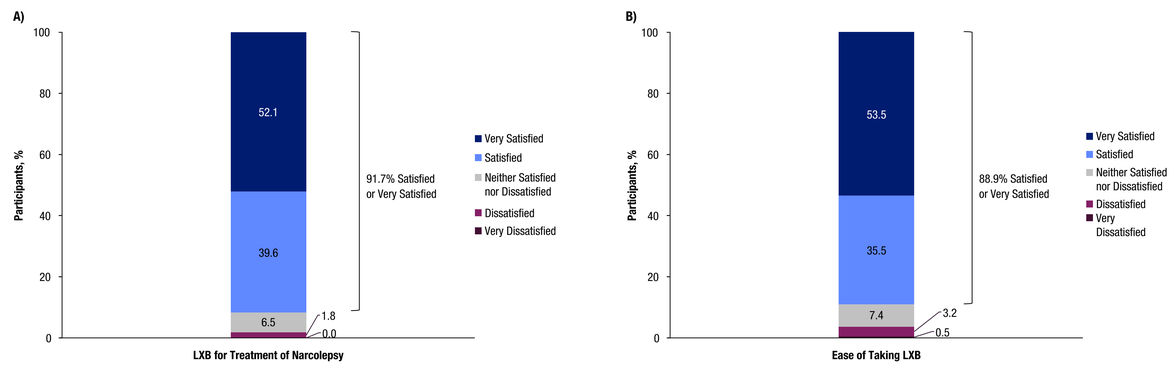
Figure 5. (A) Importance of Individualized Dosing With LXB[a] and (B) Ease of Adherence to Dosing Regimen[b]



[a]Participants were asked, "How important to you is it that, in consultation with your healthcare provider, your LXB doses can be adjusted based on your individual needs and/or experience?" [b]Participants were asked, "How easy or difficult is it for you to adhere to your LXB dosing regimen?"
LXB, low-sodium oxybate.

- 88.0% of participants reported that it was "very important" or "extremely important" that their LXB doses could be adjusted in consultation with their provider based on their individual needs and/or experience
- 89.4% of participants reported that it was "somewhat easy" or "very easy" to adhere to their LXB dosing regimen

Figure 6. (A) Overall Satisfaction With LXB for Treatment of Narcolepsy[a] and (B) Ease of Taking LXB[b]



[a]Participants were asked, "Overall, how satisfied are you with LXB for your narcolepsy?" [b]Participants were asked, "How satisfied are you with how easy it is to take LXB?"
LXB, low-sodium oxybate.

- 91.7% of participants reported that they were "satisfied" or "very satisfied" with LXB for treating their narcolepsy
- 88.9% of participants reported that they were "satisfied" or "very satisfied" with how easy it was to take LXB

References: 1. Xywav® (calcium, magnesium, potassium, and sodium oxybates) oral solution, CIII [prescribing information]. Palo Alto, CA: Jazz Pharmaceuticals, Inc.; 2023. **2.** Szarfman A, et al. *N Engl J Med.* 1995;333(19):1291. **3.** US Food and Drug Administration. Clinical review for Binosto, NDA 202344. 2012. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/nda/2012/202344Orig1s000MedR.pdf. **4.** US Food and Drug Administration. Quantitative labeling of sodium, potassium, and phosphorus for human over-the-counter and prescription drug products. Guidance for industry. 2022. Available at: https://www.fda.gov/regulatory-information/search-fda-guidance-documents/quantitative-labeling-sodium-potassium-and-phosphorus-human-over-counter-and-prescription-drug.

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