Oxybate Treatment Patterns in Patients With Narcolepsy: Cohort Data From Mayo Clinic and Duke Health

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INTRODUCTION

- Narcolepsy is a rare, chronic sleep disorder characterized by excessive daytime sleepiness (EDS) that affects approximately 30-40 per 100,000 people in the United States and 50 per 100,000 people in the European Union[1-4]
- Other symptoms of narcolepsy include disturbed or disrupted nocturnal sleep (DNS) and rapid eye movement–related phenomena (RRP), including sleep paralysis, sleep-related hallucinations, and, in people with narcolepsy type 1 (NT1), cataplexy[4]
- Sodium oxybate (SXB) is strongly recommended by both the 2021 American Academy of Sleep Medicine and European clinical practice guidelines for the treatment of EDS and cataplexy in narcolepsy[5,6]
- The US Food and Drug Administration (FDA) has approved 3 oxybate (OXB) medications for the treatment of cataplexy or EDS in patients 7 years of age and older with narcolepsy:
  - 2 immediate-release, twice-nightly OXB formulations (SXB and calcium, magnesium, potassium, and sodium [mixed-salt] OXBs)[7,8]
  - Extended-release, once-nightly SXB (ON-SXB)[9]
- In addition to cataplexy and EDS, research also suggests that ON-SXB may have an effect on DNS and the RRP sleep paralysis and sleep-related hallucinations in patients with NT1[10-12]
- A previous analysis of electronic health record data from the Mayo Clinic found that only 8% (351/4387) of patients with narcolepsy were treated with SXB, despite being approved by the FDA since 2002[13]
  - Including additional data from a Duke Health cohort builds on this prior research from Mayo Clinic by incorporating a more racially diverse patient population, thereby enhancing generalizability and inclusivity of findings

OBJECTIVE

- To characterize the demographic characteristics and concomitant medication use of patients with narcolepsy treated or not treated with OXB medications

METHODS

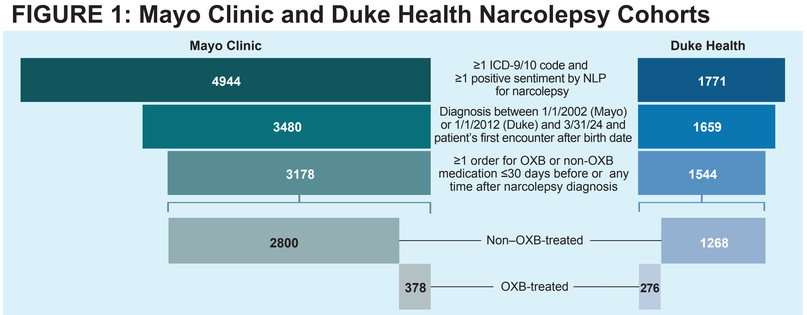
- A retrospective, electronic health record–based search identified first-time patients from Mayo Clinic and Duke Health between 2002-2024 and 2012-2024, respectively
- Data were included from patients who had
  - ≥1 narcolepsy-specific *International Classification of Diseases, 9th Revision* (ICD-9; 347.*) or *10th Revision* (ICD-10; G47.4*) code AND
  - ≥1 diagnostic mention of narcolepsy in clinical notes, identified using a natural language processing (NLP) algorithm
- Patients who received OXB or non-OXB medication ≤30 days before or any time after narcolepsy diagnosis were identified
- Narcolepsy subtype was identified as follows:
  - NT1: ICD-9/10 codes of "narcolepsy with cataplexy" and/or positive NLP sentiment for "cataplexy"
  - Narcolepsy type 2 (NT2): ICD-9/10 codes of "narcolepsy without cataplexy"
- All data were stratified by OXB use (ie, patients who had received OXB medications [OXB-treated] and those who had received medications other than OXB [non-OXB-treated]) and analyzed descriptively
- Race (Black or African American, white or Caucasian, and other), sex, narcolepsy subtype (NT1 or NT2), age at the time of diagnosis and treatment, and concomitant medication use were evaluated

RESULTS

PATIENT DEMOGRAPHICS

- A total of 4722 patients with narcolepsy were identified (Mayo, n=3178; Duke, n=1544; **Figure 1**)
  - 654 (14%) patients received OXB (Mayo, n=378; Duke, n=276), and 4068 (86%) patients received non-OXB medications (Mayo, n=2800; Duke, n=1268)

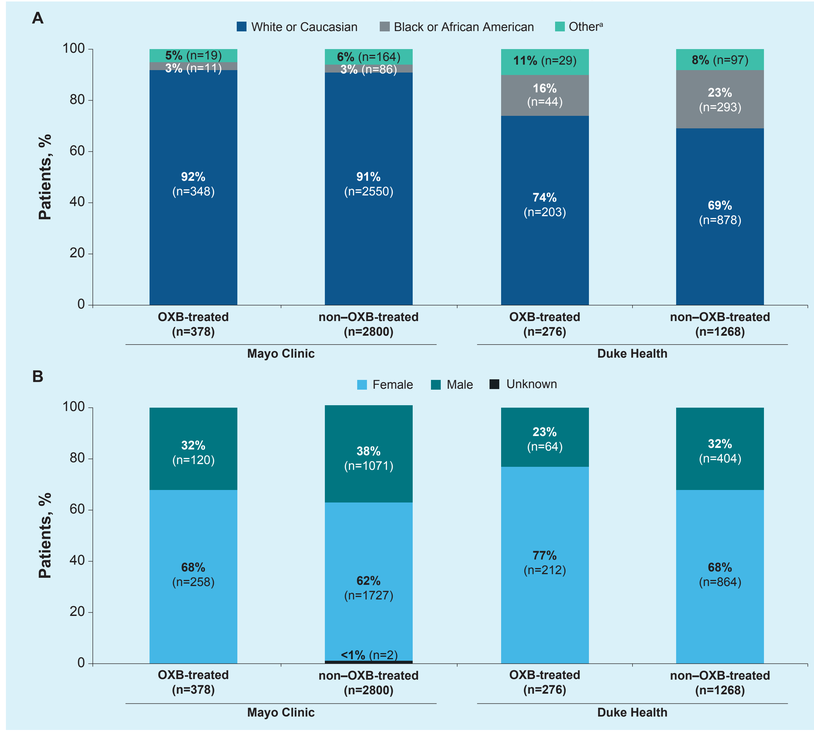
FIGURE 1: Mayo Clinic and Duke Health Narcolepsy Cohorts



ICD, *International Classification of Diseases*; NLP, natural language processing; OXB, oxybate.

- The majority of patients were white (Mayo, 91%-92%; Duke, 69%-74%) and female (Mayo, 62%-68%; Duke, 68%-77%; **Figure 2**)
  - In both Mayo Clinic and Duke Health cohorts, there was a higher proportion of female patients in the OXB-treated group than in the non–OXB-treated group

FIGURE 2: Patient Distribution by (A) Race and (B) Sex

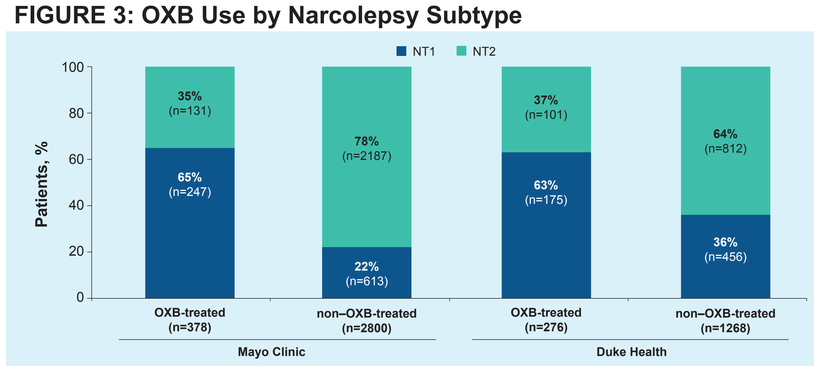


OXB, oxybate.
[a]Other race includes Asian, Native American or Pacific Islander, mixed race (Duke Health cohort only), and unknown.

NARCOLEPSY TREATMENT PATTERNS

- Most (Mayo, 65%; Duke, 63%) OXB-treated patients had NT1; the majority (Mayo, 78%; Duke, 64%) of non–OXB-treated patients had NT2 (**Figure 3**)

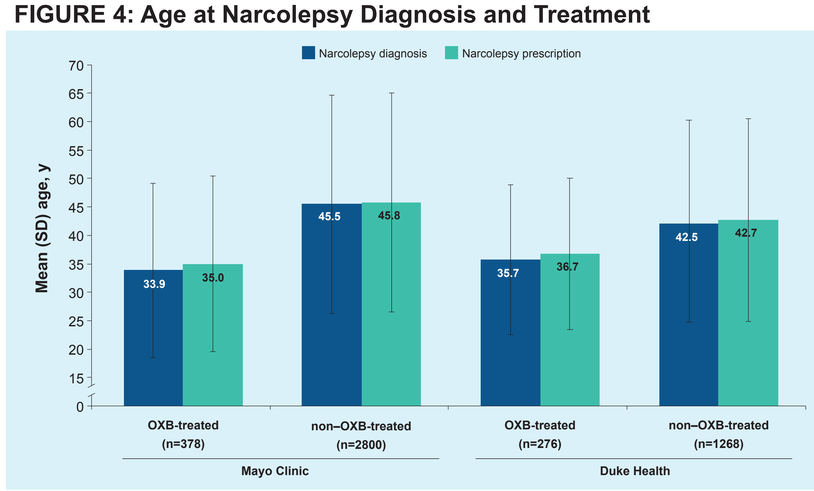
FIGURE 3: OXB Use by Narcolepsy Subtype



NT1, narcolepsy type 1; NT2, narcolepsy type 2; OXB, oxybate.

- In both the Mayo Clinic and Duke Health cohorts, mean age at diagnosis was lower for OXB-treated patients compared with non–OXB-treated patients (**Figure 4**)
- Time from diagnosis to first prescription of any narcolepsy treatment was similar between Mayo Clinic (OXB-treated, 1.1 y; non–OXB-treated, 0.3 y) and Duke Heath (OXB-treated, 1.0 y; non–OXB-treated, 0.2 y)

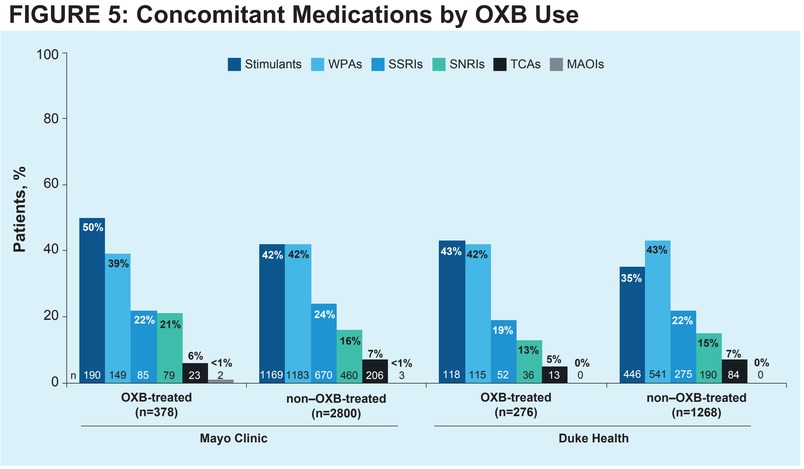
FIGURE 4: Age at Narcolepsy Diagnosis and Treatment



OXB, oxybate.

- Across both cohorts, the most common concomitant medications were stimulants and wakefulness-promoting agents, with similar medication use patterns observed between OXB-treated and non–OXB-treated groups (**Figure 5**)

FIGURE 5: Concomitant Medications by OXB Use



Concomitant medications were those prescribed ±60 days from an OXB or non-OXB treatment for narcolepsy after diagnosis. Patient counts are not mutually exclusive; not every patient may have concomitant medication use or a patient may take ≥1 type of concomitant medication.
Stimulants: lisdexamfetamine, methylphenidate, mixed salts/amphetamines, dexmethylphenidate, serdexmethylphenidate, and dextroamphetamine; WPAs: armodafinil, modafinil, pitolisant, and solriamfetol; SSRIs: citalopram, escitalopram, fluoxetine, paroxetine, and sertraline; SNRIs: desvenlafaxine, duloxetine, levomilnacipran, and venlafaxine; TCAs: amitriptyline, amoxapine, atomoxetine, clomipramine, desipramine, doxepin, imipramine, nortriptyline, protriptyline, trimipramine, and viloxazine; MAOIs: isocarboxazid, phenelzine, selegiline, and tranylcypromine.
MAOI, monoamine oxidase inhibitor; OXB, oxybate; SNRI, selective norepinephrine reuptake inhibitor; SSRI, selective serotonin reuptake inhibitor; TCA, tricyclic antidepressant; WPA, wakefulness-promoting agent.

STUDY LIMITATIONS

- The differences between the OXB-treated and non–OXB-treated groups were similar between institutions; however,
  - The Mayo Clinic cohort included a less racially diverse patient population than the Duke Health cohort
  - Characteristics evaluated were limited to race, sex, narcolepsy subtype, age at diagnosis and treatment, and concomitant medication use; other demographic or clinical characteristics may provide additional insight into OXB treatment patterns in people with narcolepsy
  - Although the addition of NLP algorithms to detect cataplexy likely increased the number of patients identified with NT1, diagnosis of NT1 may still have been underestimated if cataplexy was not documented

CONCLUSIONS

- In this retrospective analysis of nearly 5000 patients from Mayo Clinic and Duke Health, approximately 1 in 8 received OXB medications
  - The ~1-year delay from diagnosis to first treatment in both OXB-treated groups warrants further analysis
- While SXB has been approved for >2 decades and is strongly recommended for treatment of both EDS and cataplexy in narcolepsy, OXB utilization was substantially greater in patients with NT1 vs those with NT2
- As oxybate can be used to treat narcolepsy symptoms for both people with NT1 and NT2, further OXB-related education remains critical

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DISCLOSURES

MCL has no disclosures to report. **MS** has served on advisory boards for Jazz Pharmaceuticals. **AS** was an employee of nference. **JG** was an employee of Avadel Pharmaceuticals and is a consultant to Alkermes, Inc. **LEK** has served as a consultant and/or on advisory boards for Avadel Pharmaceuticals, Harmony Biosciences, and Takeda Pharmaceutical Co.

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