Demographic and Clinical Characteristics of People Receiving Once-Nightly Sodium Oxybate Treatment: A Specialty Pharmacy Data Analysis

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INTRODUCTION

- Narcolepsy is a rare, chronic sleep disorder that is classified as narcolepsy type 1 (NT1) or narcolepsy type 2 (NT2)[1]
- The main symptoms of narcolepsy include excessive daytime sleepiness (EDS), disturbed or disrupted nocturnal sleep, and rapid eye movement–related phenomena (ie, cataplexy [NT1-specific], sleep-related hallucinations, and sleep paralysis)[1,2]
  - EDS has been recognized as one of the narcolepsy symptoms with the largest impact on patients' lives, for both those with NT1 and NT2[3]
- There are no curative treatments for narcolepsy, and patients require lifelong symptom management with therapeutics such as stimulants, alerting/wake-promoting agents, and/or oxybates (OXBs)[4,5]
- Twice-nightly OXBs (TN-OXBs) include sodium oxybate (SXB; Xyrem® [sodium oxybate] oral solution, Jazz Pharmaceuticals) and mixed-salt OXBs (Xywav® [calcium, magnesium, potassium, and sodium oxybates] oral solution, Jazz Pharmaceuticals)[6,7]
- Once-nightly SXB (ON-SXB; LUMRYZ® [sodium oxybate] for extended-release oral suspension, Avadel Pharmaceuticals), which was initially approved for the treatment of EDS or cataplexy in adults with narcolepsy in May 2023 and subsequently approved for patients 7 years with narcolepsy in October 2024,[8-10] has demonstrated safety and efficacy in treating narcolepsy symptoms[11-13]

OBJECTIVE

- To assess real-world demographic and clinical characteristics, and Epworth Sleepiness Scale (ESS) data at baseline, as well as treatment adherence, in patients with narcolepsy who received ON-SXB from a single specialty pharmacy

METHODS

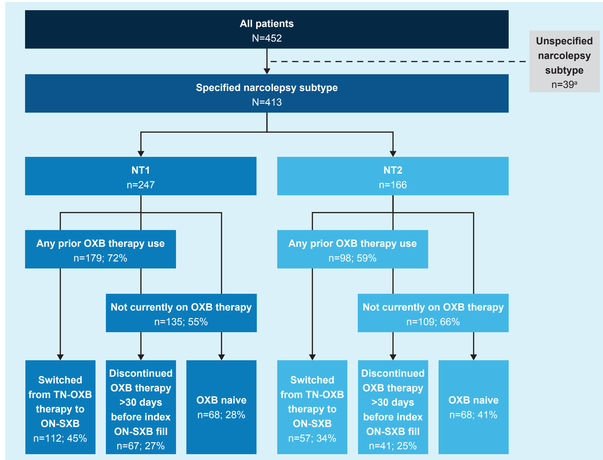
- Baseline demographic and clinical characteristic data were collected from Optum Frontier Therapies specialty pharmacy over an approximate 16-month period from June 1, 2023 to September 30, 2024 for patients who had:
  - ≥1 filled ON-SXB prescription
  - ≥1 ON-SXB-related clinical assessment
  - A documented baseline ESS total score
- Index date: first ON-SXB prescription fill during the study period
- If a discontinuation date was not reported, patients with a gap of ≥60 days in ON-SXB therapy were considered to have discontinued treatment
- Patients included in this analysis had a specified narcolepsy subtype (ie, NT1 or NT2) as determined by diagnosis code at time of index ON-SXB fill
- Prior OXB therapy use status categories were defined as:
  - Switched from prior TN-OXB to ON-SXB ≤30 days of index (ie, switch)
  - Discontinued OXB >30 days before index ON-SXB fill (ie, discontinued)
  - Had never previously received an OXB therapy (ie, OXB naive)
- For patients with ≥3 months of ON-SXB pharmacy fill data during the study period, proportion of days covered (PDC) was used to assess patient refill behavior over time as a surrogate for adherence (ie, PDC ≥80% of days covered)
  - PDC was calculated by dividing the number of days that patients had possession of ON-SXB by the number of days from the index date to the follow-up end date
    - The follow-up end date varied amongst patients and, unless reported by the patient or provider, was considered to be the last fill date plus the number of supply days
- All data were analyzed descriptively; no statistical testing was performed

RESULTS

BASELINE DEMOGRAPHICS AND CLINICAL CHARACTERISTICS

- Of 452 identified patients, 413 had a specified narcolepsy subtype; the majority had been diagnosed with NT1 (NT1, n=247 [60%]; NT2, n=166, [40%]; **Figure 1**)
  - Of the 244 (59%) patients who were not currently on OXB therapy (ie, not-on-OXB patients; NT1, n=135; NT2, n=109), 108 (NT1, n=67; NT2, n=41) had discontinued prior OXB >30 days before index and 136 (NT1 and NT2, n=68 each) were OXB naive

FIGURE 1: Patient Population by Prior OXB Therapy Use Status Across Narcolepsy Subtypes



Percentages were calculated based on the total number of patients within each narcolepsy subtype.
NT1, narcolepsy type 1; NT2, narcolepsy type 2; ON-SXB, once-nightly sodium oxybate; OXB, oxybate; TN-OXB, twice-nightly oxybate.
[a]A total of 39 patients (9%) did not have a specified narcolepsy subtype and were excluded from further analyses.

- Mean (SD) age was 38.3 (14.4) years and 39.2 (13.0) years for patients with NT1 and NT2, respectively (**Table 1**)
- The majority of patients were female (NT1, 71%; NT2, 67%) and had commercial insurance (NT1, 77%; NT2, 81%)
- Of 277 patients with prior OXB therapy use (NT1, n=179; NT2, n=98), mixed-salt OXBs was the most common medication (NT1, 61%; NT2, 70%)

TABLE 1: Baseline Demographics and Clinical Characteristics

Characteristic	NT1 (n=247)	NT2 (n=166)
Age, mean (SD), y	38.3 (14.4)	39.2 (13.0)
Sex, n (%)		
Female	175 (71)	111 (67)
Male	72 (29)	55 (33)
Type of prior OXB therapy medication, n (%)[a]		
Mixed-salt OXBs	109 (61)	69 (70)
SXB	77 (43)	34 (35)
Insurance type, n (%)		
Commercial	191 (77)	134 (81)
Medicare or Medicaid	41 (17)	21 (13)
Missing/Unknown	15 (6)	11 (7)
ESS total score, mean (SD)	11.7 (5.5)	12.5 (5.3)

ESS, Epworth Sleepiness Scale; NT1, narcolepsy type 1; NT2, narcolepsy type 2; OXB, oxybate; SXB, sodium oxybate.
[a]Among patients with any prior OXB use (n=277). Twelve (NT1, n=7; NT2, n=5) patients had previously received SXB and mixed-salt OXBs.

MEDICATION USE AT BASELINE

- Alerting/wake-promoting agents and/or stimulants were the most frequently prescribed medications, regardless of narcolepsy subtype (NT1, 64%; NT2, 65%; **Table 2**)

TABLE 2: Baseline Medication Use

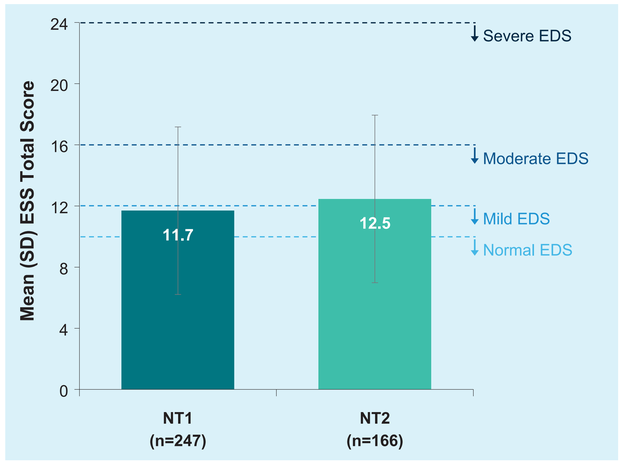
Medication, n (%)	NT1 (n=247)	NT2 (n=166)
Alerting/wake-promoting agents and/or stimulants	159 (64)	108 (65)
Alerting/wake-promoting agents[a]	118 (48)	73 (44)
Stimulants	82 (33)	61 (37)
Amphetamine, short-acting[b]	38 (15)	33 (20)
Amphetamine, long-acting[c]	30 (12)	26 (16)
Methylphenidate, short-acting[d]	21 (9)	8 (5)
Methylphenidate, long-acting[e]	6 (2)	6 (4)
Antidepressants[f]	108 (44)	68 (41)
Antihypertensives[g]	59 (24)	48 (29)

NT1, narcolepsy type 1; NT2, narcolepsy type 2.
[a]Includes modafinil, armodafinil, and soriamfetol; pitolisant was also captured in this category. [b]Includes amphetamine, amphetamine sulfate, dextroamphetamine, dextroamphetamine sulfate, mixed amphetamine salts, and methamphetamine HCl. [c]Includes amphetamine, dextroamphetamine sulfate, mixed amphetamine salts, and lisdexamfetamine dimesylate. [d]Includes methylphenidate HCl and dexmethylphenidate HCl. [e]Includes methylphenidate HCl, dexmethylphenidate HCl, and serdexmethylphenidate/dexmethylphenidate. [f]Includes antidepressants (atypical), anxiolytics, general anesthetics (nonbarbiturates), monoamine oxidase inhibitors, selective serotonin reuptake inhibitors, serotonin and norepinephrine reuptake inhibitors, and tricyclic antidepressants. [g]Includes angiotensin-converting enzyme inhibitors, angiotensin II receptor antagonists, beta blockers, calcium channel blockers, thiazide and thiazide-like diuretics, aldosterone receptor antagonists, alpha blockers, centrally acting alpha-2 adrenergic agonists, loop diuretics, potassium-sparing diuretics, direct renin inhibitors, and vasodilators.

ESS TOTAL SCORES AND SEVERITY OF EDS AT BASELINE

- Mean (SD) ESS total score at baseline was 11.7 (5.5) in patients with NT1 and 12.5 (5.3) in those with NT2 (**Table 1**)
  - Mean ESS scores were in the range of mild EDS for NT1 and moderate EDS for NT2[14,15] (**Figure 2**)

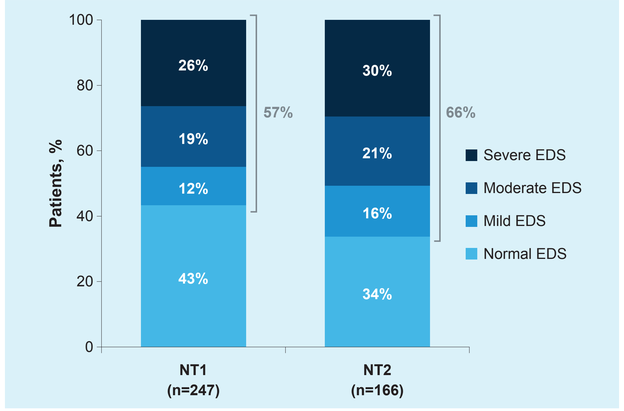
FIGURE 2: Baseline ESS Total Scores



Normal, mild, moderate, and severe EDS defined as ESS total score of ≤10, 11-12, 13-15, and ≥16, respectively.[14,15]
EDS, excessive daytime sleepiness; ESS, Epworth Sleepiness Scale; NT1, narcolepsy type 1; NT2, narcolepsy type 2.

- At baseline, 66% of patients with NT2 and 57% of those with NT1 experienced mild-to-severe EDS (**Figure 3**)

FIGURE 3: Baseline Severity of EDS

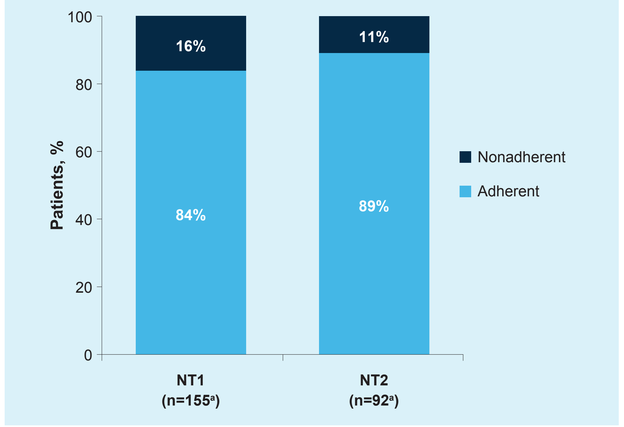


Normal, mild, moderate, and severe EDS defined as ESS total score of ≤10, 11-12, 13-15, and ≥16, respectively.[14,15]
EDS, excessive daytime sleepiness; ESS, Epworth Sleepiness Scale; NT1, narcolepsy type 1; NT2, narcolepsy type 2.

ON-SXB TREATMENT ADHERENCE

- Median (IQR) follow-up was 152 (62-252) days and 120 (53-214) days for patients with NT1 and NT2, respectively
- Within the first 3 months of ON-SXB use, 88% of patients with NT1 and 86% of those with NT2 took a maintenance dose of 6 g, 7.5 g, or 9 g
- Of 247 patients with ON-SXB use for ≥3 months (NT1, n=155; NT2, n=92), adherence rates, as inferred from refill behavior, were similar between narcolepsy subtypes (NT1, 84%; NT2, 89%; **Figure 4**)
- Mean (SD) PDC was also similar for NT1 (91% [15%]) and NT2 (92% [12%])

FIGURE 4: ON-SXB Treatment Adherence Rates



ON-SXB adherence based on pharmacy fill data, with adherent defined as PDC ≥80% of days covered and nonadherent defined as PDC <80% of days covered.
NT1, narcolepsy type 1; NT2, narcolepsy type 2; ON-SXB, once-nightly sodium oxybate; PDC, proportion of days covered.
[a]Patients with ≥3 months of ON-SXB use during the study period.

STUDY LIMITATIONS

- These data were limited to patients who received ON-SXB through a single specialty pharmacy of the 4 that dispense ON-SXB, which may restrict generalizability to broader narcolepsy patient populations
- Given that these data were derived from prescription fills, actual adherence to the prescribed regimen cannot be confirmed
- As this was a descriptive analysis, statistical significance was not assessed to formally compare results between NT1 and NT2 groups

CONCLUSIONS

- This analysis provides data on baseline characteristics for patients who initiated ON-SXB shortly after its initial approval, across an approximately 16-month period
- At baseline, the NT1 and NT2 subgroups were categorized as having mild and moderate EDS, respectively, based on mean ESS total score
- Prior to their first ON-SXB prescription fill during the study period, patients with NT1 were prescribed OXBs more frequently than those with NT2 despite comparable or greater sleepiness burden in patients with NT2
- Adherence rates among patients with ≥3 months of ON-SXB treatment during the study period were similar between narcolepsy subtypes

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DISCLOSURES

CMR has served as an advisory board member for Alkermes, Inc., Eisai, Jazz Pharmaceuticals, and Takeda Pharmaceutical Co. and has received grant funding from Jazz Pharmaceuticals.

MJT has served as a consultant or on advisory boards for Axsome Therapeutics, Balance Therapeutics, Avadel Pharmaceuticals, Eisai, Harmony Biosciences, Jazz Pharmaceuticals, NLS Pharmaceutics, Suven Life Sciences Ltd., and Takeda Pharmaceutical Co.

LBH has participated in clinical research for Alkermes, Inc., Axsome Therapeutics, Avadel Pharmaceuticals, Breas Medical, Centessa Pharmaceuticals, Eisai, Fisher & Paykel Healthcare, Harmony Biosciences, Idorsia, Jazz Pharmaceuticals, Lilly, LivaNova/OSPREY, Merck & Co., Noctrix Health, Samsung, Sanofi, Suven Life Sciences Ltd., Takeda Pharmaceutical Co., and Vanda Pharmaceuticals and served as speaker or consultant for Avadel Pharmaceuticals, Fisher & Paykel Healthcare, Harmony Biosciences, Idorsia, and Jazz Pharmaceuticals.

SM has served on an advisory board for Avadel Pharmaceuticals and has received research funding from Alkermes, Inc., Avadel Pharmaceuticals, Axsome Therapeutics, and Jazz Pharmaceuticals.

MV and **EB** are employees of Optum Life Sciences and stockholders of UnitedHealth Group.

DC is an employee of Optum Frontier Therapies and a stockholder of UnitedHealth Group.

BA is an employee of Alkermes, Inc.

JG was an employee of Avadel Pharmaceuticals and is currently a consultant to Alkermes, Inc.

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