

# Real-World Healthcare Resource Utilization and Economic Burden of Narcolepsy Type 1 in the US

ISPOR 2026

May 17 – 20, 2026  
Philadelphia, PA, USA

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## Key Objectives

- To characterize the demographics, comorbidities, healthcare resource use, and costs among the NT1 population using medical claims data
- To quantify the incremental clinical and economic burden of NT1 relative to controls by comparing comorbidities and costs between these populations
- To quantify the incremental clinical and economic burden of NT1 relative to NT2 by comparing comorbidities, costs, and medication use between these populations

## Introduction

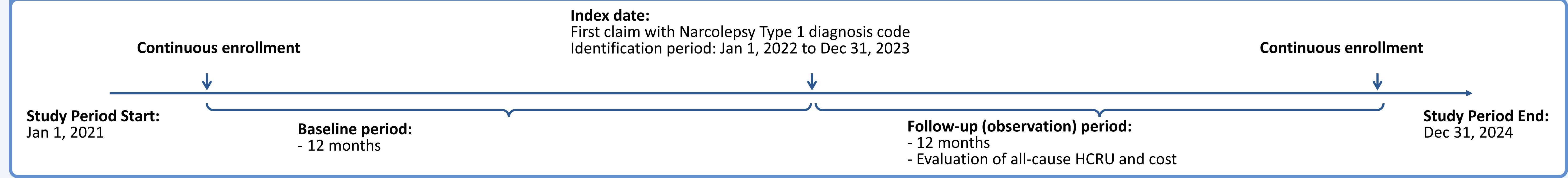
- Narcolepsy is a chronic neurologic condition that causes dysregulation of the sleep-wake cycle and is characterized by a pentad of symptoms: excessive daytime sleepiness, cataplexy, hypnagogic hallucinations, sleep paralysis, and disrupted nocturnal sleep<sup>1</sup>
- Narcolepsy is further categorized as<sup>1</sup>:
  - Narcolepsy Type 1 (NT1) – narcolepsy with cataplexy
  - Narcolepsy Type 2 (NT2) – narcolepsy without cataplexy
- Cataplexy is a sudden, transient loss of muscle tone usually triggered by strong emotion<sup>2</sup>
  - Attacks typically last seconds to minutes, occur with preserved consciousness, and may range from partial cataplexy (where one or more of the limbs or the head and neck are involved) to complete cataplexy (where there is whole body atonia leading to complete bodily collapse)<sup>2</sup>
- Previous studies have explored the burden of narcolepsy<sup>3</sup>
  - However, the relative clinical and economic burden of NT1 and NT2 have not been studied

## Methods

### Study Design

- This was a retrospective, longitudinal, observational study (Jan 1, 2021 to Dec 31, 2024) using pharmacy and medical administrative claims data from the MarketScan Commercial and Medicare Supplemental database (Figure 1)
- The study comprised of patients who were adults at the index date and had  $\pm 12$  months of continuous enrollment
- Patients were sorted into three cohorts and inclusion/exclusion criteria for each are described below:
  - NT1 cohort:** Two ICD-10-CM claims for narcolepsy with cataplexy (G47.411 or G47.421)  $\geq 30$  days apart from Jan 1, 2022 to Dec 31, 2023. The date of the first ICD-10-CM claim with NT1 diagnosis was defined as the index date
  - NT2 cohort:** Two ICD-10-CM claims for narcolepsy (G47.419 or G47.429)  $\geq 30$  days apart from Jan 1, 2022 to Dec 31, 2023, but were not part of the NT1 cohort. The date of the first ICD-10-CM claim with a narcolepsy diagnosis was defined as the index date
  - Controls cohort:** No ICD-10-CM claims for narcolepsy (G47.411, G47.419, G47.421, or G47.429). A random index method was used to evaluate inclusion/exclusion based on age and continuous enrollment  $\pm 12$  months
    - Due to notable differences with regards to demographic characteristics between NT1 and controls, a matched control cohort was identified based on age ( $\pm 5$  years), sex, index year, region, payer type, and insurance plan type to reduce the imbalance between NT1 and controls

Figure 1. Study Design



### Outcomes

- Patient demographics and comorbidities during the 12 months pre-index period
- All-cause costs, all-cause healthcare resource use (HCRU), and use of key medications of interest (pitolisant, oxybates, and oxybates/stimulants<sup>a</sup>) during the 12-month post-index period

### Statistical Analyses

- Outcomes were evaluated in the NT1 population descriptively, and compared between NT1 vs NT2 cohorts and NT1 vs matched controls cohorts using t-test for continuous variables and chi-squared tests for categorical variables
  - To evaluate the impact of key psychiatric comorbidities of interest (anxiety, depression, or attention deficit hyperactivity disorder), a sensitivity analysis was conducted evaluating all-cause costs in patients with NT1 without key psychiatric comorbidities of interest vs patients with NT2 without key psychiatric comorbidities of interest

<sup>a</sup>Oxybates or stimulants that were classified as Drug Enforcement Administration schedule II/III medications.

## Results

Table 1. Demographic and Clinical Characteristics

	Narcolepsy Type 1 (n = 1707)	Narcolepsy Type 2 (n = 4981)	Matched Controls (n = 6811)
Age, years, mean (SD)	41.6 (15.2)	44.1 (14.7)	41.6 (15.2)
Female, %	70%	64%	70%
US geographic region, %			
Northeast	11%	10%	11%
North Central	29%	32%	29%
South	49%	50%	50%
West	10%	8%	10%
Payer Type, %			
Commercial	93%	93%	93%
Medicare Supplemental	7%	7%	7%
Plan type <sup>a</sup> , %			
HMO	9%	10%	9%
PPO	54%	54%	54%
HDHP	13%	13%	13%
Elixhauser Comorbidity Index <sup>a</sup> , mean (SD)	2.1 (2.2)	2.0 (2.2)	1.2 (1.8)
Comorbidities of interest			
ADHD	12%	11%	4%
Anxiety	37%	36%	19%
Asthma	12%	11%	5%
COPD	2%	3%	1%
Chest Pain	14%	12%	7%
Depression	29%	30%	11%
Diabetes	10%	11%	8%
Hypercholesterolemia	23%	27%	20%
Hypertension	28%	28%	20%
Nausea	11%	9%	6%
Obesity	24%	24%	14%
Sleep Apnea	33%	33%	5%

- The study sample comprised of 1707 individuals with NT1, 4981 individuals with NT2, and 6811 matched controls (Table 1)

- In NT1 cohort, the mean Elixhauser Comorbidity Index score was 2.1 and most common baseline comorbidities were anxiety (37%), sleep apnea (33%), depression (29%), and hypertension (28%). Baseline characteristics were similar in the NT2 cohort
- Compared to matched controls, the NT1 cohort had numerically higher prevalence of comorbidities despite similar demographic characteristics

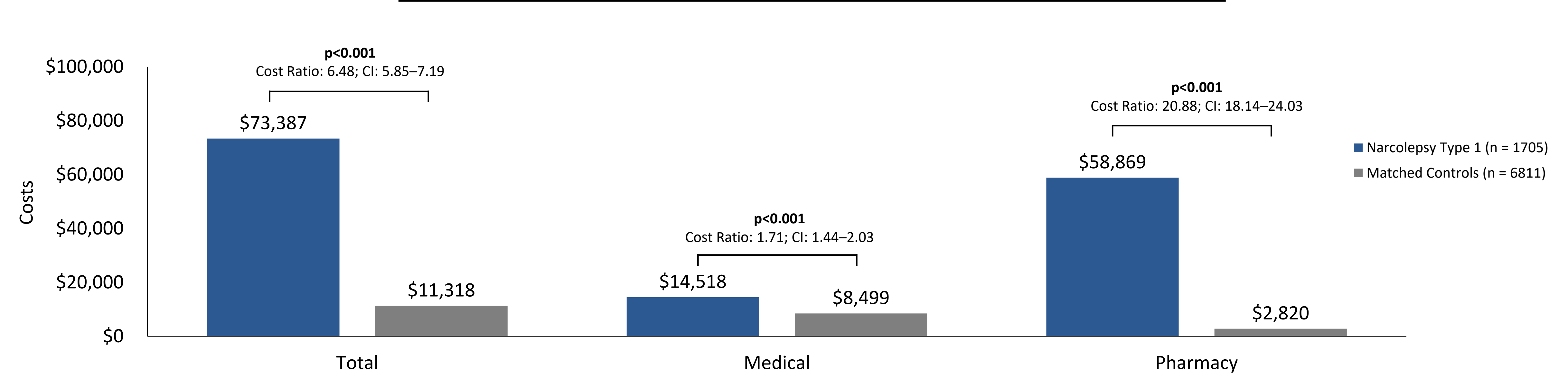
<sup>a</sup>The Elixhauser Comorbidity Index is a risk-adjustment measure used to identify multiple pre-existing conditions using ICD diagnosis codes which are combined into weighted summary scores. Higher weighted summary scores indicate greater comorbidity burden and worse expected outcomes  
ADHD, attention deficit hyperactivity disorder; COPD, chronic obstructive pulmonary disorder; HDHP, high-deductible health plan; HMO, Health Maintenance Organization; PPO, Preferred Provider Organization.

### NT1 Cohort: Costs and HCRU During 12-months Post-index

- Mean annual all-cause total, medical, and pharmacy costs in the NT1 cohort were \$73,486, \$14,514, and \$58,972, respectively
- A total of 7% of individuals in the NT1 cohort had a  $\geq 1$  hospitalization visit and 27% had  $\geq 1$  emergency room visit
  - Among those with  $\geq 1$  hospitalization, average cost of hospitalization was \$53,193 and 11% had a re-admission within 30 days
- Individuals in the NT1 cohort had a mean number of 24.42 outpatient visits and 37.34 pharmacy fills, and 64% were prescribed Drug Enforcement Administration schedule II/III medications
- Polypharmacy occurred in 23% of patients in the NT1 cohort

### NT1 vs Control Cohort: All-cause Costs and Psychiatric Comorbidities During the 12-Months Post-index Period

Figure 2. Healthcare Costs in the NT1 and Control Cohorts Over the 12 Months Post-Index Period<sup>a</sup>

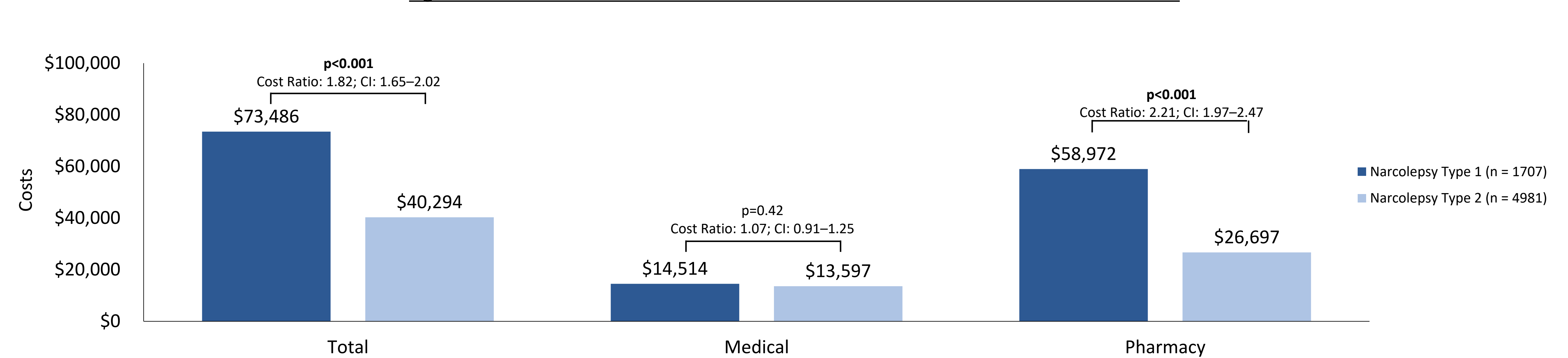


- Compared with the matched controls cohort, the NT1 cohort had higher total annual costs (cost ratio 6.48;  $p < 0.001$ ; Figure 2), which were primarily driven by pharmacy costs (cost ratio 20.88;  $p < 0.001$ )
- Individuals in the NT1 cohort were more likely to experience anxiety or depression compared with matched controls (odds ratio: 3.12;  $p < 0.001$ ; CI: 2.78 – 3.49)

<sup>a</sup>Two NT1 patients were excluded from the analysis since no matches were identified among controls.

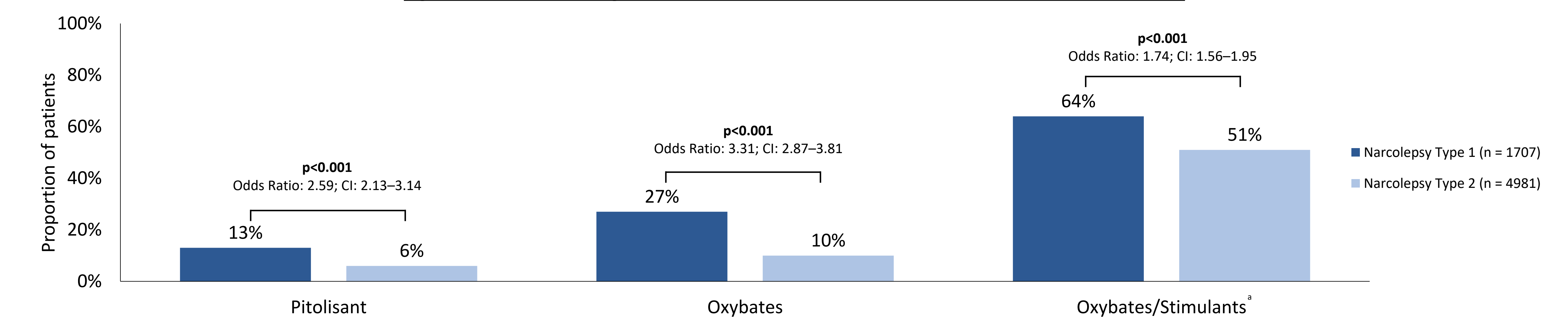
### NT1 vs NT2 Cohorts: All-cause Costs and Medication Usage During the 12-Months Post-index Period

Figure 3. Healthcare Costs in the NT1 and NT2 Cohorts Over the 12 Months Post-Index Period



- Compared with the NT2 cohort, the NT1 cohort had higher total annual costs (cost ratio: 1.82;  $p < 0.001$ ; Figure 3), driven primarily by pharmacy costs (cost ratio: 2.21;  $p < 0.001$ )
- In a sensitivity analysis comprising individuals with NT1 without anxiety, depression, or attention deficit hyperactivity disorder, the costs were approximately 80% of the costs in the overall NT1 population; mean total costs continued to be approximately twofold higher compared with individuals with NT2 without anxiety, depression, or attention deficit hyperactivity disorder (\$61,316 [n=881] vs. \$34,483 [n=2531];  $p < 0.001$ ; cost ratio: 1.78; CI: 1.54–2.06)

Figure 4. Medication Usage in the NT1 and NT2 Cohorts Over the 12 Months Post-Index Period



- Compared with individuals with NT2, individuals with NT1 were more likely to be prescribed pitolisant (odds ratio 2.59;  $p < 0.001$ ; Figure 4), oxybates (odds ratio 3.31;  $p < 0.001$ ), or oxybates/stimulants<sup>a</sup> (odds ratio: 1.74;  $p < 0.001$ )

<sup>a</sup>Oxybates or stimulants that were classified as Drug Enforcement Administration schedule II/III medications.

## References

- Bassetti CLA, Adamantidis A, Burdakov D, et al. *Nat Rev Neurol*. 2019;15(9):519-539.
- Dauvilliers Y, Siegel JM, Lopez R, et al. *Nat Rev Neurol*. 2014;10(7):386-395.
- Thorpy MJ, Hiller G. *Am Health Drug Benefits*. 2017;10(5):233-241.

## Acknowledgments

This study was funded by Axsome Therapeutics, Inc. Medical writing and editorial support was provided by Bassaam Mulk, Pharm.D, of Axsome Therapeutics, Inc.

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## Disclosures

DT Plante has served as a consultant and/or advisory board member for Aditum Bio, LLC, Alkermes, Apnimed, Centessa, Harmony Biosciences, Jazz Pharmaceuticals, Takeda, and Teva Pharmaceuticals (Australia). W Wang and J Cong employees of KMK Consulting. S Floam and L Bessonova are employees of Axsome Therapeutics.



## Limitations

- General limitations of observational claims database studies include the potential for incomplete/missing data or data entry and/or coding errors
- Analyses were not adjusted for potential confounders
- Results from this study may not be generalizable to other patient populations

## Conclusions

- NT1 is commonly associated with psychiatric and metabolic/cardiometabolic comorbidities
  - Patients with NT1 exhibit a high number of annual pharmacy fills and outpatient visits as well as costly inpatient stays
  - Annual healthcare costs for patients with NT1 are approximately six times those of controls
  - Patients with NT1 incur roughly twice the healthcare costs, regardless of psychiatric comorbidities, compared with patients with NT2
    - Additionally, use of Drug Enforcement Administration schedule II/III medications (oxybates and stimulants) was higher among patients with NT1 compared with NT2
- These results highlight complex ongoing disease management due to symptoms and comorbidities, underscoring a need for range of treatment options to help balance effectiveness and tolerability