EE138

Treatment Patterns Among People Newly Diagnosed With Narcolepsy Type 1 in the United States: A Real-World Data Study

Stephen Crawford,[1] Brian Calingaert,[2] Juliana Meyers,[2] LaStella Sinclair,[2] Amy Duhig,[1] Sean Candrilli,[2] Christine Ulbricht[1]

[1]Takeda Development Center Americas, Inc., Cambridge, MA, US; [2]RTI Health Solutions, 3040 E Cornwallis Rd, Durham, NC, US

Background

- Narcolepsy type 1 (NT1) is a rare, chronic neurological disorder of hypersomnolence characterized by excessive daytime sleepiness, cataplexy, hallucinations, sleep paralysis, and disrupted nighttime sleep[1,2]
- Current NT1 treatments do not completely control excessive daytime sleepiness and cataplexy, and often multiple medications are required due to limited efficacy or tolerability[3]
- There is a high disease burden and large unmet need for adequate treatment options for individuals with NT1[3]
- We present the demographic characteristics and real-world treatment patterns of individuals newly diagnosed with NT1 in the United States (US)

Objective

- To describe demographics and clinical characteristics of individuals newly diagnosed with NT1
- To describe real-world treatment patterns among individuals newly diagnosed with NT1

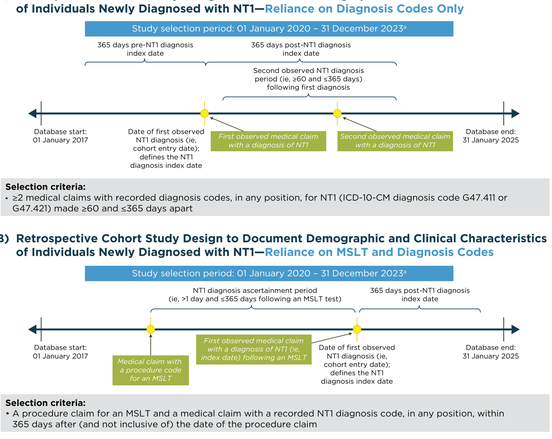
Methods

Study Design

- This was a retrospective observational study in adults, including commercially insured and Medicare Supplemental beneficiaries in the US newly diagnosed with NT1 using the Merative MarketScan Commercial Claims and Encounters (CCAE) and Medicare Supplemental and Coordination of Benefits databases (MDCR) from January 2017 to January 2025 (**Figure 1**)
- All individuals with NT1 in the MarketScan CCAE and MDCR databases were identified based on one or more of the following selection criteria during the study selection period (01 January 2020-31 December 2023)
  - ≥2 medical claims with recorded diagnosis codes, in any position, for NT1 (ICD-10-CM diagnosis codes G47.411 or G47.421) ≥60 and ≤365 days apart (**Figure 1A**)

    OR
  - A procedure claim for a multiple sleep latency test (MSLT) and a medical claim with a recorded NT1 diagnosis code within 365 days after the date of the procedure claim (**Figure 1B**)
- The NT1 diagnosis index date was defined as the date of the first observed NT1 diagnosis during the selection window
- Individuals with an NT1 diagnosis prior to the NT1 diagnosis index date identified during the study selection period were excluded to ensure the study was limited to individuals with newly diagnosed NT1 during the study period

Figure 1. Study design criteria based on (A) diagnosis codes only (B) MSLT and diagnosis codes



Note: Figure not drawn to scale. aData from 01 January 2017 through 31 December 2019 were used to rule out prevalent cases and individuals with certain comorbid conditions (ie, Kleine-Levin syndrome, myotonic dystrophy type 1, or idiopathic hypersomnia).
The NT1 diagnosis index date was defined as the date of the first qualifying diagnosis of NT1 during the selection window.
MSLT, multiple sleep latency test; NT1, narcolepsy type 1.

Outcomes

- Demographic and clinical characteristics on the NT1 diagnosis index date
- Comorbidities observed in the 365 days before the NT1 diagnosis index date
- Proportion of individuals receiving medications for symptomatic treatment in the 365 days before the NT1 diagnosis index date and medications used to treat NT1 in the 365 days after the NT1 diagnosis index date
  - Narcolepsy-indicated treatments (NITs) are treatments indicated, per the label, for narcolepsy, including sodium oxybate, pitolisant, modafinil, armodafinil, and solriamfetol
  - Narcolepsy-related treatments (NRTs) are treatments commonly used to treat narcolepsy (eg, stimulants and antidepressants) that are not indicated, per the label, for the treatment of narcolepsy
- The date of first observed receipt of a treatment indicated, per the label, for narcolepsy defined the treatment index date
- Index treatment regimen was defined as the first observed treatment indicated, per the label, for narcolepsy
- Treatment discontinuation, time to discontinuation, treatment switching and augmentation in the 365 days after the treatment index date
- Medication adherence (medication possession ratio [MPR]), persistence [in days])
- Polypharmacy (overlapping use of ≥2 treatments, with treatments assessed including NRTs and NITs)

Data Analysis

- Study measures and outcomes were analyzed descriptively

Results

Participants

- A total of 580 individuals with newly diagnosed NT1 were included, of whom 71.7% were female
- The median (IQR) age was 33 (23-45) years (**Table 1**)
- Over 95% of individuals had commercial insurance and the most common health plan types were preferred provider organizations (55.2%), high-deductible health plans (11.9%), or health maintenance organizations (11.2%) (**Table 1**)

Table 1. Demographics of NT1 cohort

Characteristic	Individuals newly diagnosed with NT1
Total sample (N)	580
Age on NT1 diagnosis index date, median years (IQR)	33 (23-45)
Distribution, n (%)	
18-24	176 (30.3)
25-34	138 (23.8)
35-44	113 (19.5)
45-54	83 (14.3)
55-64	52 (9.0)
65-74	5 (0.9)
75+	13 (2.2)
Geographic region, n (%)	
South	307 (52.9)
North Central	151 (26.0)
Northeast	63 (10.9)
West	57 (9.8)
Missing/Unknown	2 (0.3)
Insurance type, n (%)	
Commercial	561 (96.7)
Medicare supplemental	19 (3.3)
Health plan type, n (%)	
Preferred provider organization	320 (55.2)
High-deductible health plan	69 (11.9)
Health maintenance organization	65 (11.2)
Consumer-driven health plan	61 (10.5)
Point of service	32 (5.5)
Comprehensive	22 (3.8)
Exclusive provider organization	4 (0.7)
Missing/unknown	7 (1.2)

NT1, narcolepsy type 1.

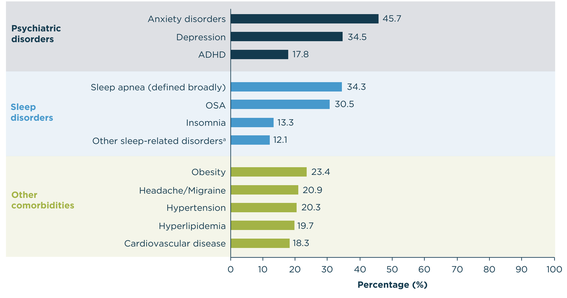
Diagnosis Pattern

- In the 365 days before the NT1 diagnosis index date, 46.7% of individuals had ≥1 diagnostic test
- The most common diagnostic tests were polysomnography (31.0%), MSLT/maintenance of wakefulness test (21.4%), and brain computed tomography/MRI (18.4%). Human leukocyte antigen genotyping (1.0%) and cerebrospinal fluid testing (0.0%) were uncommon

Comorbidities

- The mean (SD) Charlson Comorbidity Index was 1.2 (1.5) in the 365 days before the NT1 diagnosis date in individuals newly diagnosed with NT1
- The most common psychiatric comorbidities observed in the 365 days before the NT1 diagnosis index date were anxiety disorders (45.7%), depression (34.5%), and attention-deficit/hyperactivity disorder (17.8%). The most common comorbid sleep disorders were sleep apnea (34.3%), obstructive sleep apnea (30.5%), and insomnia (13.3%) (**Figure 2**)

Figure 2. Comorbidities during the 365 days prior to the NT1 diagnosis index date

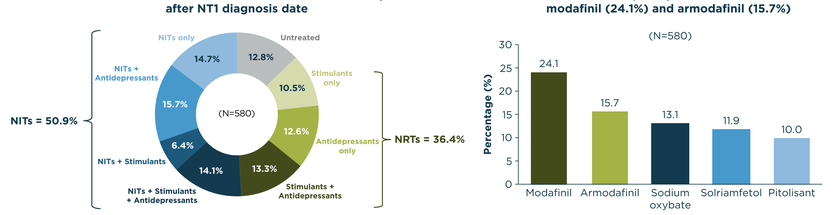


[a]Includes restless legs syndrome, sleep fragmentation, periodic limb movement disorders, rapid eye movement sleep behavior disorder, excessive fragmentary myoclonus, other sleep-related movement disorders.
ADHD, attention-deficit/hyperactivity disorder; NT1, narcolepsy type 1; OSA, obstructive sleep apnea.

Treatment Patterns

- During the 365 days prior to their NT1 diagnosis index date, 51.7% received antidepressants, 31.0% received stimulants, 27.2% received anxiolytics/hypnotics, and 25.3% received mood stabilizers
- Within 365 days after the NT1 diagnosis index date, 87.2% (506/580) received NRTs (stimulants, antidepressants) or NITs (sodium oxybate, pitolisant, modafinil, armodafinil, and solriamfetol), while 12.8% remained untreated (**Figure 3A**)
- Overall, 14.7% received only NITs, 14.1% received NITs along with stimulants and antidepressants, while 13.3% received only stimulants and antidepressants in the 365 days after their NT1 diagnosis index date (**Figure 3B**)
- Over half of individuals newly diagnosed with NT1 received NITs any time in the 365 days after their NT1 diagnosis index date (295/580; **Figure 3A**), most commonly modafinil (24.1%) and armodafinil (15.7%) (**Figure 3C**)

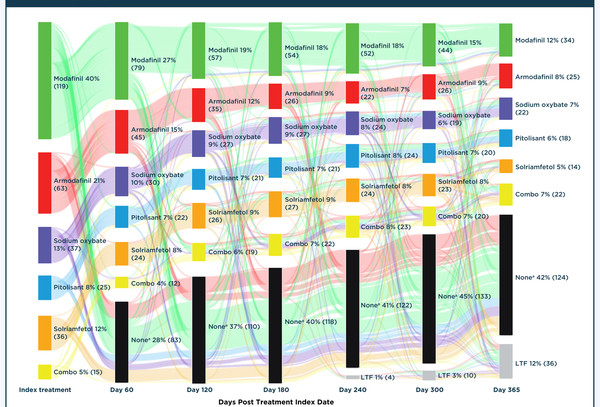
Figure 3. Treatment patterns in the 365 days after the NT1 diagnosis index date



[a]Those on NITs could also be on NRTs concurrently; those on polypharmacy were on both. Polypharmacy percentages in the figure are based on the total population rather than the treated population only.
NIT, narcolepsy-indicated treatments; NRT, narcolepsy-related treatment; NT1, narcolepsy type 1.

- Among individuals who received only NITs (N=295) modafinil was the most common medication initiated on the index treatment date (40%) and remained the most common narcolepsy-indicated treatment within 365 days of the index treatment date (**Figure 4**)
  - By day 365, 42% were not receiving any of the 5 NITs (**Figure 4**)

Figure 4. Receipt of 5 narcolepsy-indicated treatments in the 365 days after the index treatment date

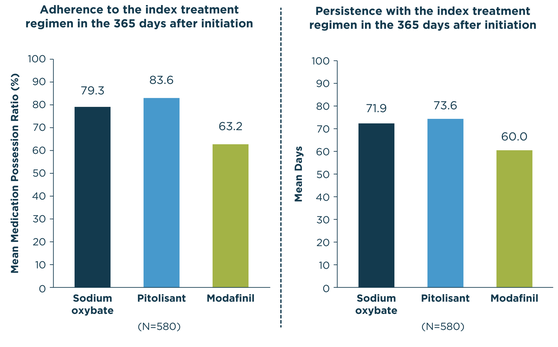


[a]None indicates that the individuals in the group were not taking any of the 5 narcolepsy-indicated treatments; however, they could be taking other treatments.
Combo, combination of 2 or more of the 5 narcolepsy-indicated treatments; LTF, loss to follow-up due to end of enrollment; NT1, narcolepsy type 1.

Adherence and Persistence

- Mean MPR and persistence were 83.6% and 73.6 days for pitolisant, 79.3% and 71.9 days for sodium oxybate, and 63.2% and 60.0 days for modafinil, respectively (**Figure 5**).

Figure 5. Adherence and persistence to the index treatment regiment among individuals newly diagnosed with NT1



MPR, medication possession ratio; NT1, narcolepsy type 1.

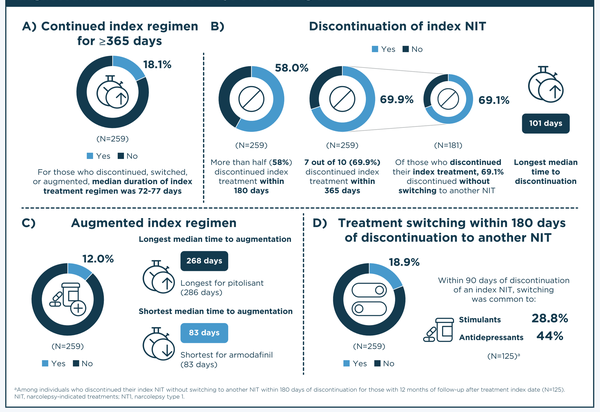
Polypharmacy Among Individuals Newly Diagnosed With NT1

- Polypharmacy was common among individuals newly diagnosed with NT1
  - Among all 506 treated individuals, 56.7% received ≥2 NRTs or NITs simultaneously
  - Of the 295 individuals receiving NITs, 23.4% received ≥2 simultaneously

Treatment Initiation, Discontinuation, Augmentation, and Switching

- Among individuals with 365 days of follow-up after index NIT (n=259), the mean (SD) time to initiation of the index NIT was 71.8 (85.4) days
- Discontinuation of the index NIT reached 69.9% (181/259) within 365 days after treatment initiation, while 18.1% continued their index treatment for ≥365 days (**Figure 6A** and **B**); 69.1% (125/181) of those who discontinued their index NIT discontinued without switching to another NIT medication
  - Among patients with 12 months of follow-up after the index date, 48.3% (125/259) discontinued the index NIT regimen without switching to another NIT within 180 days of discontinuation
  - Of these, 44% (55/125) switched to an antidepressant and 28.8% (36/125) switched to a stimulant within 90 days of discontinuing the index treatment regimen
- Augmentation of the index NIT occurred in 12.0% (31/259; **Figure 6C**); treatment switching to another of the 5 NITs occurred within 180 days among 18.9% (49/259) of individuals (**Figure 6D**), with a mean (SD) of 81.1 (69.7) days from the treatment index date

Figure 6. Treatment augmentation, discontinuation, and switching among individuals newly diagnosed with NT1 within 365 days of receiving an index NIT



[a]Among individuals who discontinued their index NIT without switching to another NIT within 180 days of discontinuation for those with 12 months of follow-up after treatment index date (N=125).
NIT, narcolepsy-indicated treatments; NT1, narcolepsy type 1.

Limitations

- The MarketScan data represent a subset of the insured population in the United States and thus findings might not be generalizable to other insured populations or to populations without insurance
- This retrospective claims-based study relied on billing codes, which are subject to misclassification; some individuals may not have truly had NT1, although requiring 2 NT1 diagnoses 60-365 days apart helped reduce misclassification
- Because NT1 could have been diagnosed prior to database start and NT1 symptoms can fluctuate, the true disease onset date may not have been accurately identified, potentially misclassifying some prevalent cases as incident cases
- The study could not determine the reason medications were prescribed and whether medications were taken as prescribed
- Claims data lack clinical detail and mortality information, limiting assessment of disease severity and outcomes

Conclusions

- **The findings of this study indicate that only about half of individuals newly diagnosed with NT1 received narcolepsy indicated treatments within 365 days after diagnosis, with treatment initiation often delayed following diagnosis**
- **With moderate persistence and adherence and high discontinuation rates, changes in treatment regimens were frequent**
- **Many individuals required multiple narcolepsy-related medications, including antidepressants and stimulants**
- **These results highlight the ongoing unmet needs and complexity of NT1 management for those newly diagnosed**

ISPOR; May 17-20, 2026; Philadelphia, PA

References: 1. Scammell TE. *N Engl J Med.* 2015;373(27):2654-2662. **2.** Cano CA, Harel BT, Scammell TE. *Sleep.* 2024;47(9):zsae150. **3.** Barateau L, Dauvilliers Y. *Ther Adv Neurol Disord.* 2019;12:1756286419875622. **Acknowledgments:** This work was funded by Takeda Development Center Americas, Inc. Under the direction of the authors, employees of Cadent, a division of Syneos Health, provided writing and editorial assistance for this presentation. Medical writing was funded by Takeda Pharmaceuticals USA, Inc., and Takeda Development Center America, Cambridge, MA, USA. **Disclosures:** Stephen Crawford, Amy Duhig and Christine Ulbricht are employees of Takeda Development Center Americas, Inc., Cambridge, MA, US. Brian Calingaert, Juliana Meyers, LaStella Sinclair, and Sean Candrilli are employees of RTI Health Solutions, Durham, NC, US.

Scan the QR code to access the poster and any additional materials on your mobile device as well as to forward yourself a link to it via email. If you do not have a QR reader, please enter https://tiny.one/rsp2564Or1 in your browser