# Real-world Treatment Utilization of Sodium Oxybate in Pediatric Patients With Narcolepsy: An Analysis of Claims Data

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Chad M. Ruoff, MD¹; Aatif M. Husain, MD²; Sheila Reiss Reddy, PhD, RPh³; Ryan S. Tieu, MS⁵; Danielle Hyman, PhD⁴; Morgan Bron, PharmD, MS⁵; Kathleen F. Villa, MS⁵

<sup>1</sup>Stanford Center for Sleep Sciences and Medicine, Palo Alto, CA, USA; <sup>2</sup>Duke University Medical Center, Duke University Medical Center, Duke Clinical Research Institute, and Veterans Affairs Medical Center, Duke University Medical Center, Duke Univers

## Background

- Narcolepsy is a rare, lifelong, neurological disorder characterized by excessive daytime sleepiness and dysregulation of the sleep-wake cycle, including cataplexy, hypnagogic hallucinations, and sleep paralysis, though not all patients experience all symptoms<sup>1</sup>
- Sodium oxybate (SXB) is considered a standard of care for the treatment of narcolepsy<sup>2</sup>
- In pediatric narcolepsy clinical trials, median SXB compliance rates were very high, ranging from 83% to 100% for up to 47 weeks<sup>3,4</sup>
- However, real-world SXB utilization in pediatric patients has not been well studied

## **Objective**

• To examine SXB adherence, persistence, and overall treatment utilization patterns in real-world settings in pediatric patients with narcolepsy in the United States

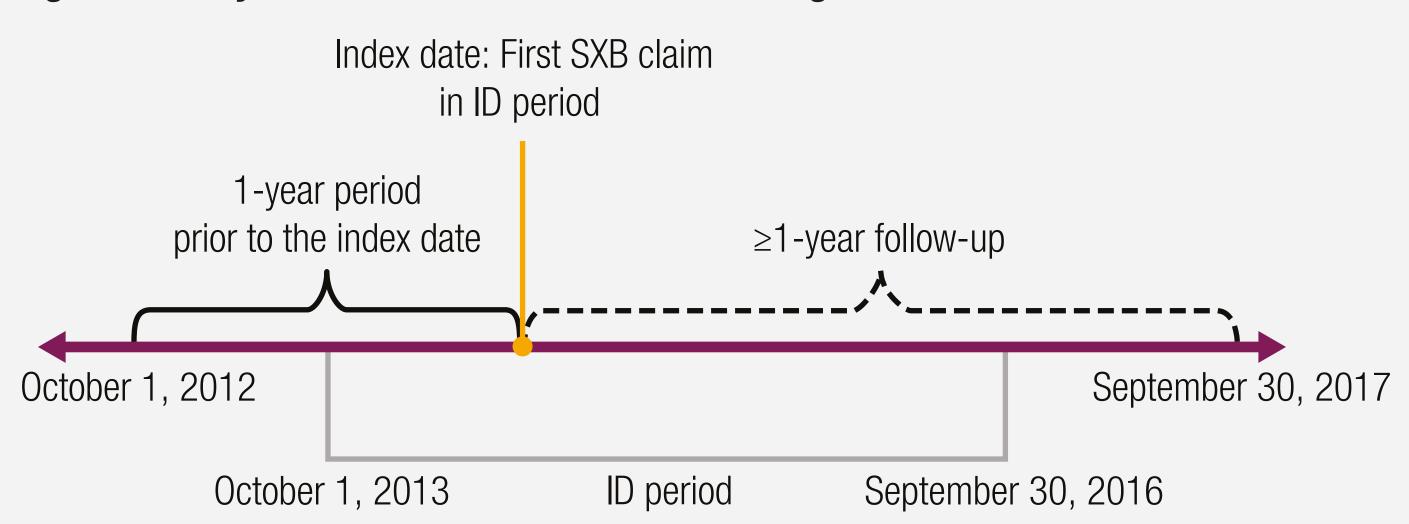
## Methods

- This retrospective cohort study identified US patients <18 years of age diagnosed with narcolepsy using de-identified commercial insurance claims from the Truven Health Analytics MarketScan® Commercial Claims and Encounters database from October 2012 through September 2017
- Patients with narcolepsy were first identified based on having ≥1 of the following: ≥1 nondiagnostic claim with a code for narcolepsy with or without cataplexy and ≥1 claim for SXB; ≥2 claims with a code for narcolepsy (1 being nondiagnostic); or ≥1 nondiagnostic claim with a code for narcolepsy following a Multiple Sleep Latency Test (MSLT)

 Nondiagnostic claims were defined as claims for evaluation and management services without MSLT/polysomnography or other diagnostic testing

- For the analysis of SXB treatment patterns, narcolepsy patients who were being treated with ≥1 claim for SXB during the identification (ID) period (October 1, 2013-September 30, 2016) were identified
- The first claim for SXB during the ID period was defined as the index date Patients were required to have continuous enrollment for ≥1 year prior to and ≥1 year after the index date, with ≥1 nondiagnostic claim prior to the index date
- New users were defined as those who did not have any SXB claims during the 1-year period prior to the index date; continuing users were defined as those who had ≥1 SXB claim during the 1-year period prior to the index date

#### Figure 1. Study Timeline for New Versus Continuing SXB Users



- SXB, sodium oxybate; ID, identification.
- Treatment utilization, adherence, and persistence of SXB were measured at 3 months, 6 months, 9 months, 1 year, and 2 years

 Adherence was assessed as mean proportion of days covered (PDC) and proportion of patients with PDC ≥80%, and persistence was evaluated by mean number of days on continuous treatment (defined as treatment with an interruption of <60 days)

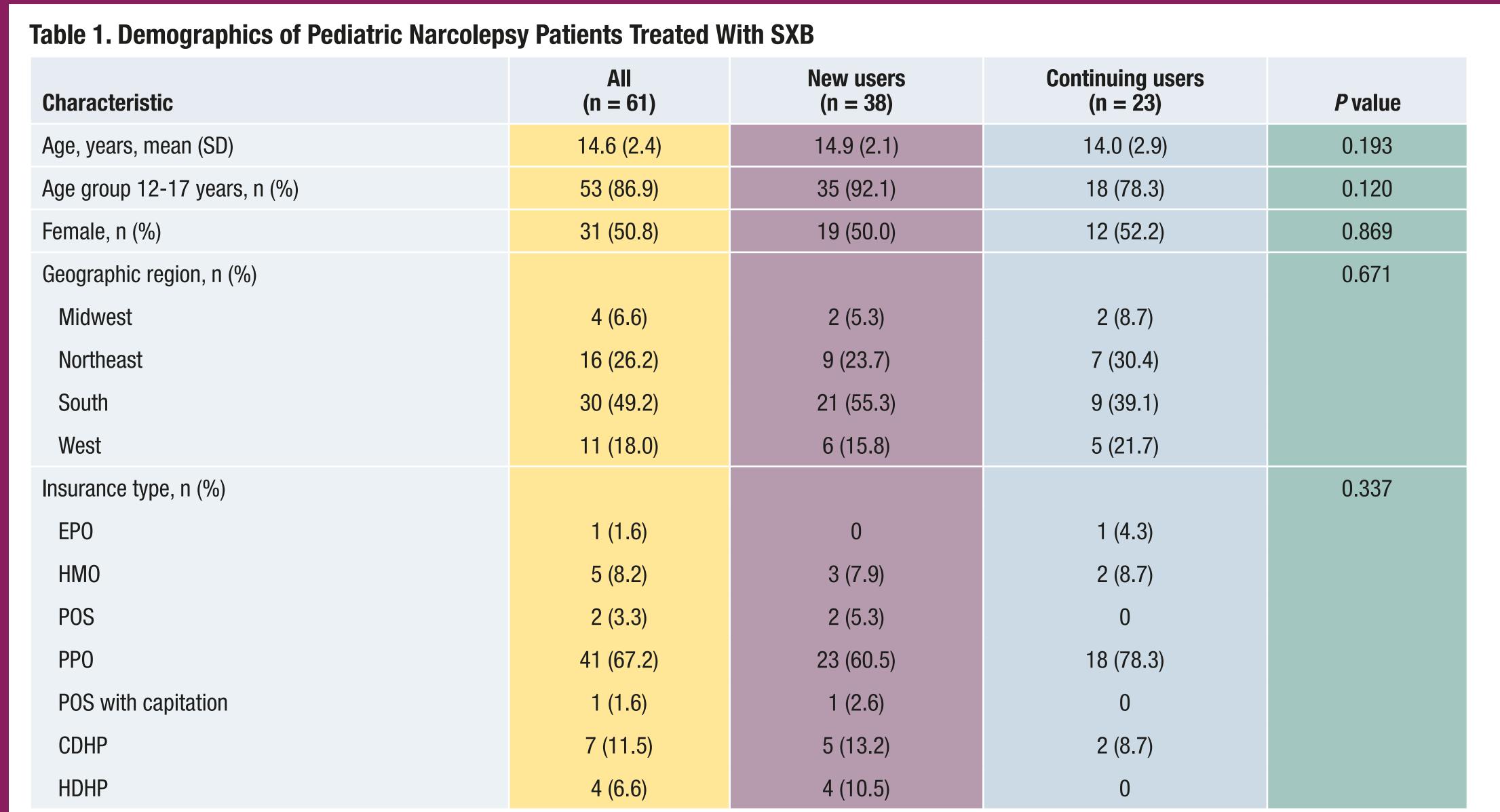
- Descriptive statistics were reported, and chi-square or t tests were performed to compare between-group treatment patterns. Kaplan-Meier curves were produced, and log-rank tests were performed to compare continuous time on treatment. P values were not controlled for multiplicity and, hence, are nominal
- Graphical analyses were performed to examine overall treatment patterns among pediatric patients with narcolepsy treated with any narcolepsy-related treatments (both indicated medications and those used to treat common comorbid conditions in patients with narcolepsy), including SXB, wake-promoting agents (WPAs), attention-deficit/hyperactivity disorder (ADHD) agents and traditional

central nervous system (CNS) stimulants, antidepressants, and nonstimulant ADHD agents The first claim for any narcolepsy-related treatment was considered the index date, with similar

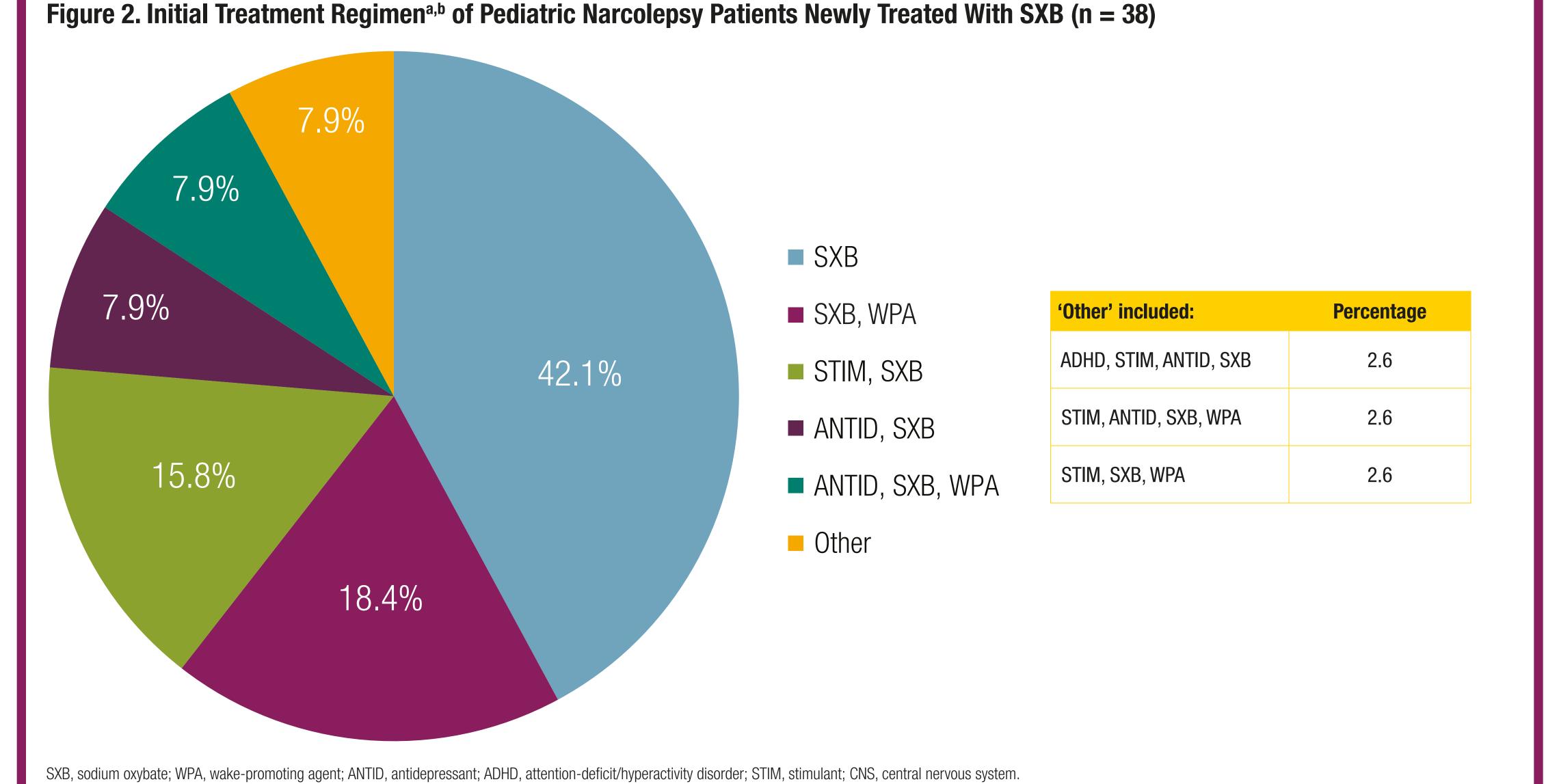
or continued index treatment (index treatment/treatment combination prior to index)

continuous enrollment requirements as described above Patients were stratified as treatment-naïve (no treatment prior to index), newly treated with index treatment (no index treatment/treatment combination prior to index but who were not treatment-naïve),

## Results

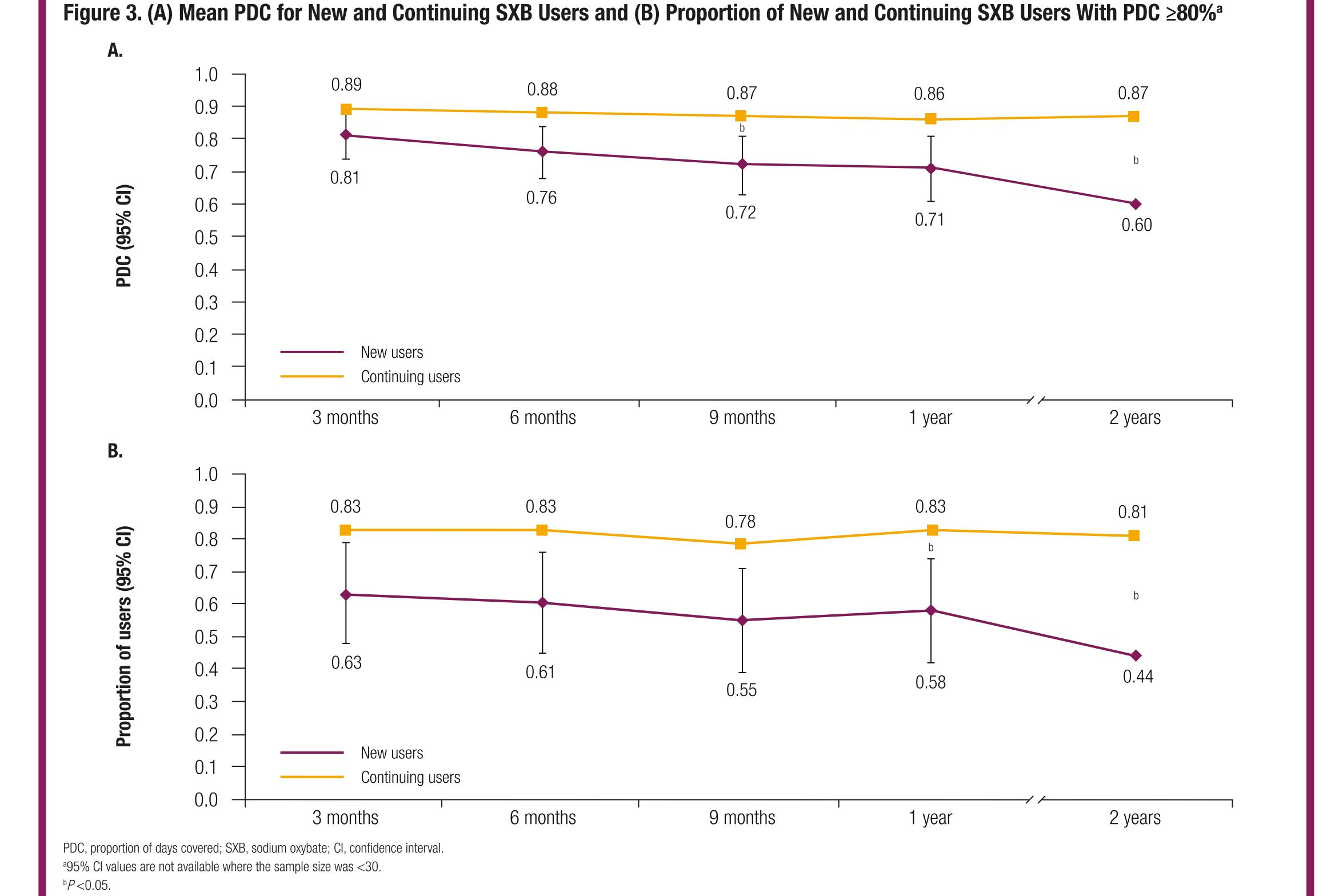


- Among 61 continuously enrolled pediatric patients taking SXB (mean [±standard deviation] age, 14.6 [±2.4] years; 50.8% female), 38 were new SXB users and 23 were continuing SXB users

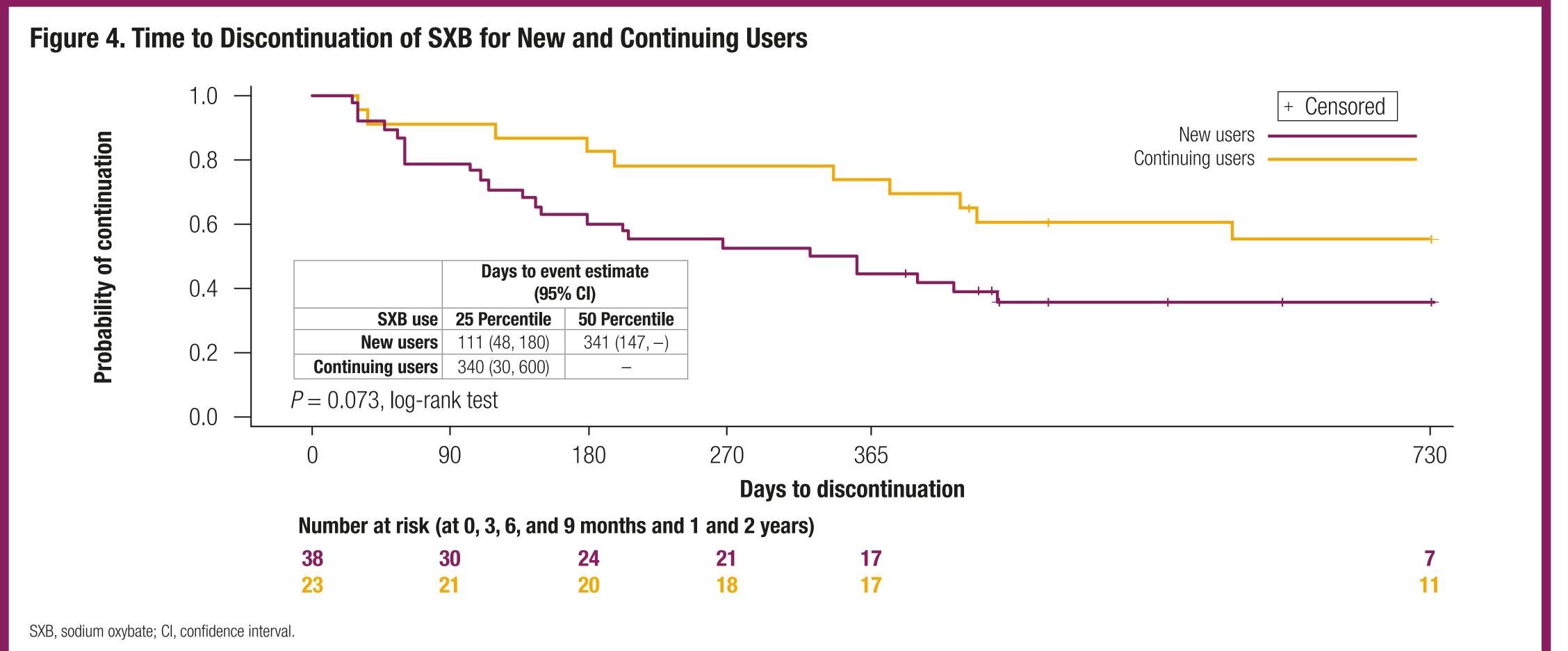


<sup>a</sup>Defined as all treatment classes occurring between the index date and 45 days after the index date.

- <sup>b</sup>Regarding treatment classes, ADHD represents nonstimulant ADHD agents, while STIM represents traditional CNS stimulants (eg, amphetamine, methylphenidate).
- Among 38 new users, 42.1% (n = 16) received monotherapy and 57.9% (n = 22) received combination therapy • Of new users receiving combination therapy (n = 22), 31.8% (n = 7) received SXB plus a WPA, 27.3% (n = 6) received SXB plus a traditional CNS stimulant, and 13.6% (n = 3) received SXB plus an antidepressant

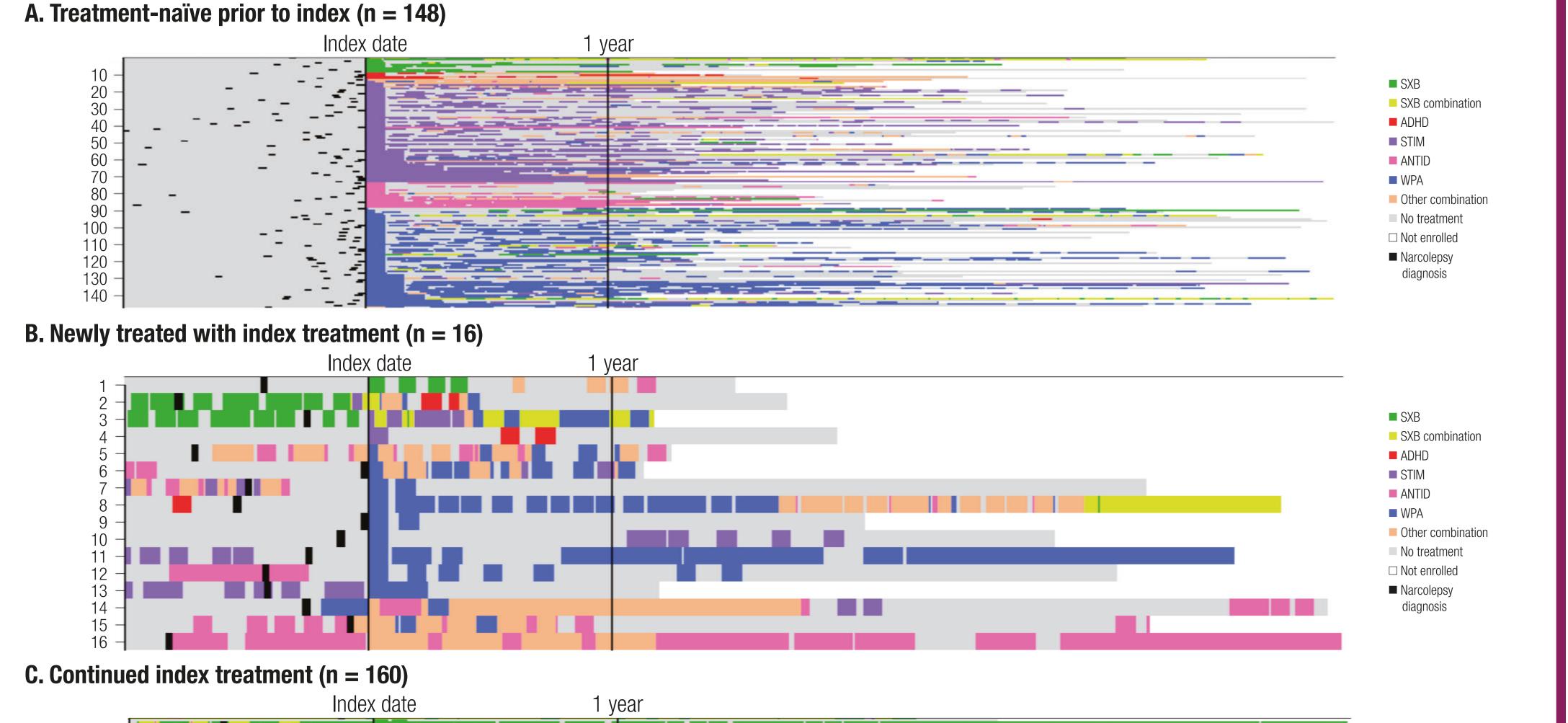


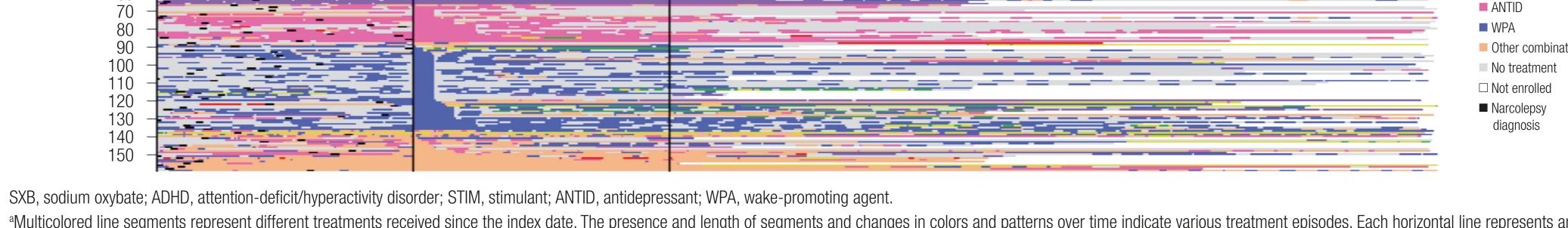




• The median (95% confidence interval) time to SXB discontinuation was 341 (147, not reached) days for new users and was not reached within the time studied for continuing users

### Figure 5. GRAPHx Patterns of Narcolepsy-related Treatment for Patients Who Were Treatment-naïve Prior to Index, Were Newly Treated With Index Treatment, and Continued Users at Index<sup>a</sup>





- individual patient's treatment history in the follow-up period, and the height of each colored section is proportional to the total number of patients receiving each treatment. Gray areas represent periods of enrollment during which there were no claims
- In all treatment groups (Figures 5A-C), interruptions in treatment use were common in the first year after the index date; however, many patients appeared to resume the treatment at the index date after an interruption, indicating persistence in use
- In contrast, switching patterns seemed infrequent across treatments at the index date (Figures 5A-C)
- Due to a very small sample size, the panel containing data for pediatric patients newly treated with index treatment (Figure 5B) could not be interpreted Adherence to the treatments at the index date (uninterrupted line segment) appeared more common among those who continued the treatment at the index date (Figure 5C) compared to treatment-naïve patients (Figure 5A), which may suggest improved tolerability or dosing adjustment after continued exposure to treatment; this pattern was sharpest among users taking SXB
- For treatment-naïve patients (Figure 5A), the most common treatment at the index date was an ADHD stimulant, followed by a WPA, an antidepressant, a nonstimulant ADHD agent, and SXB

## Limitations

- The study population primarily represents a commercially insured segment of the US population; results may not be generalizable to all patients with narcolepsy
- Claims-based analyses are limited by the possibility of diagnosis coding errors; however, concomitant requirements for MSLT and may reduce this risk

## Conclusions

- In real-world settings in the United States, SXB adherence and persistence rates were generally high in pediatric patients with narcolepsy over the 1-year assessment period
- High rates of adherence (mean PDC, 60%-89% and PDC ≥80%, 44%-83%) and persistence were observed for new and continuing SXB users relative to rates reported in the literature for other treatments<sup>5-7</sup>
- Adherence for stimulants and nonstimulants for ADHD (1-year mean medication possession ratio, 0.57 [stimulants] and 0.49 [nonstimulants])<sup>5</sup>
- Adherence for antidepressants for major depressive disorder (PDC ≥80% at 1 year, 26%)<sup>7</sup>
- Adherence rates were lower and persistence rates were similar for new versus continuing users
- These findings are consistent with data from clinical trials showing that titration to an optimal SXB dose, which may involve multiple steps, is critical in achieving an optimal response, which may take up to 2 months.<sup>4,8-10</sup> Moreover, adverse events with SXB are known to occur early and diminish over time. 10 Once optimal dosing is established as with continuing users in this study, patients can achieve and maintain long-term treatment benefits<sup>8,10-12</sup>

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