

OBJECTIVE

This analysis was done to provide an overview of current US stiripentol dosing practices for this rare syndrome, which had no specific ICD-10 diagnostic code during this period.

BACKGROUND

- Dravet syndrome is a rare, drug-resistant epilepsy syndrome that first manifests in otherwise healthy infants and is often linked to SCN1A gene mutations. It is characterized by multiple seizure types, motor deficits, intellectual disability, and behavioral problems.
- Although available in Europe since 2007, stiripentol was available in the US only on a compassionate use basis until March 2019, when it became commercially available. **Stiripentol is approved for the treatment of seizures associated with Dravet syndrome in individuals older than 2 years taking clobazam.**

METHODS

- Retrospective analysis of data from the single specialty pharmacy dispensing stiripentol from 29 April 2019 to 30 April 2020.
- All data meeting specified criteria were included in the analysis without patient identifiers; these criteria included at least 2 fills (initial and latest), gender, age, weight, prescriber location, diagnosis code, status (naïve/transition from Compassionate Use) and specific dosing instructions.

RESULTS

- Sample consisted of 740 prescriptions for 370 individuals who were 47% female [avg age = 12.5 years (± 7.2 , range 1.0-38.0) and avg weight 40.4 kg (± 1.5 ; range 9.5-113.4)]. A total of 235 were new to stiripentol and 135 transitioned from compassionate use.
- **Average dose** of the most recent prescription was **35 mg/kg/day** for naïve and **28 mg/kg/day** for transition patients. The most common **dosing frequency** was 2 times per day (76%) with 3 times (22%), once (2%), and 4 times per day (0.5%) being much less common.
- Average prescribed dose (mg/kg/day) decreased with age from 29 ± 17 at age 2 to 19 ± 10 at age 38. Overall, doses were lower than the recommended dose of 50 mg/kg/day in 2 divided doses.
- 44 different ICD-10 codes were recorded in 7 categories, the most common (93.5%) *Epilepsy and Recurrent Seizures (G40 codes)*, specifically G40.409 (45%), G40.309 (21%), and G40.319 (6.5%).

TABLE. Average Dose (mg/kg/day)

Dose in mg/kg/day Avg \pm SD (Range)	All Patients	Transition from Compassionate Use	Naïve Patients
Initial Prescription	24 \pm 14 (2 – 88)	26 \pm 15 (2 – 88)	20 \pm 12 (4 – 55)
Latest Prescription	31 \pm 17 (2 – 99)	28 \pm 17 (2 – 99)	35 \pm 17 (5 – 97)
All Prescriptions	27 \pm 16 (2-99)	27 \pm 16 (2 – 99)	28 \pm 17 (4 – 97)

KEY FINDINGS

- This analysis of prescription data from the first year of commercial availability of stiripentol, following FDA approval in 2018, includes patients new to stiripentol and those transitioning from compassionate use.
- The wide variety of ICD-10 codes reflects the lack of syndrome specific coding. **New ICD-10 codes for Dravet syndrome went into effect on 1 October 2020.**
- Although the **recommended dosing for stiripentol is 50 mg/kg/day in 2 or 3 divided doses**, averages doses were below this in this sample.
- US prescribers progressively decrease mean stiripentol dose as patient age increases, mirroring the current practice in Europe.¹