

### OBJECTIVE

This survey of US physicians treating individuals with Dravet syndrome was conducted to determine prescriptive behavior and perceived efficacy of stiripentol.

### BACKGROUND

- Dravet syndrome is a rare, genetic, encephalopathy associated with developmental delays and multiple seizure types. Stiripentol is indicated for the treatment of seizures associated with Dravet syndrome in patients 2 years and older taking clobazam.
- The recommended target dose of stiripentol is 50 mg/kg/day. This survey of US physicians using stiripentol in Dravet syndrome summarize reported prescriptive behavior and perceived efficacy.

### METHODS

- 52 physicians from 22 US centers, selected based on their experience treating patients with Dravet syndrome, were invited to complete an online survey between April and May 2020.
- 27 (52%) responded, reporting a total of 192 patients (mean of 7 patients per physician).
- Data collected included patient age group, starting dose, target dose, titration increment, titration interval, perceived percent seizure reduction, and any taper of concomitant antiepileptic drugs.

### RESULTS

- The table lists reported typical starting and target doses for pediatric and adult patients.
- Most common stiripentol titration increment was 10 mg/kg/day (38.5%). 84.1% of physicians reported titration of stiripentol at 1-week intervals.
- Reduction of concomitant clobazam dose was reported by 73% of respondents with a reduction of 25% (n=11), 50% (n=3), or other (n=5).
- 58% of respondents reported reducing concomitant valproate dose by 15% (n=2), 30% (n=5), 50% (n=1) or other (n=3).

### KEY FINDINGS

- 37% of physicians reported titrating stiripentol to at least the **recommended target dose of 50 mg/kg/day** in their pediatric patients, 7.7% reported doing so in their adult patients. (Table)
- 81% of physicians reported at least 50% seizure reduction and 31% reported 75% seizure reduction with the use of stiripentol. (Figure)**
- These US physicians are aware of the difference in pediatric versus adult stiripentol pharmacokinetics and of the usual adaptive dosing strategy for adult patients.
- The reported perceived efficacy is close to the data collected in the European pivotal, phase 3 trials and in patients in Japan.<sup>1,2</sup>

TABLE. Average Stiripentol Dose (mg/kg/day)

Dose (mg/kg/day)	PEDIATRIC		ADULT	
	Starting	Target	Starting	Target
<10	3 (11.1%)	---	1 (7.7%)	---
10	9 (33.3%)	---	3 (23.1%)	---
15	5 (18.5%)	---	1 (7.7%)	---
20	3 (11.1%)	2 (7.4%)	---	2 (15.4%)
25	1 (3.7%)	---	1 (7.7%)	---
30	---	4 (14.8%)	---	1 (7.7%)
40	---	2 (7.4%)	---	2 (15.4%)
50	---	10 (37.0%)	---	1 (7.7%)
Other	6 (22.2%)	9 (33.3%)	7 (53.8%)	7 (53.8%)
	Dosing influenced by age, efficacy, and tolerability, in addition to weight		Physicians indicated the use of fixed rather than weight-based dosing in adults	

FIGURE. Perceived Stiripentol Efficacy

