Clinical Characteristics and Treatment Patterns in Patients with Dravet Syndrome and Lennox-gastaut Syndrome Who Are Prescribed Cannabidiol (CBD)

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Rationale: Dravet syndrome (DS) and Lennox-Gastaut syndrome (LGS) are developmental and epileptic encephalopathies characterized by severe, treatment-resistant seizures and significant cognitive and developmental impairments. Limited real-world evidence exists on characteristics of patients receiving a plant-derived, highly purified, pharmaceutical formulation of CBD (Epidiolex®), approved for the treatment of seizures associated with DS and LGS. This study aimed to describe clinical characteristics, treatment patterns, and outcomes among patients in the US, UK, and EU with DS or LGS receiving CBD.

Methods: Physician-reported chart data from France, Germany, Italy, Spain, UK, and US, collected between July 2022 and August 2023 as part of the Adelphi Disease Specific Programme™ (DSP), were used. Adult and pediatric neurologists reported on demographics, clinical characteristics, co-occurring conditions, treatment history, treatment satisfaction, and reasons for prescription to patients with a confirmed diagnosis of DS/LGS prescribed CBD (Epidiolex®; 100 mg/mL oral solution). Descriptive statistics (median [Q1, Q3], %) were summarized.

Results: Data were provided by 88 neurologists for 191 patients prescribed CBD at time of survey (DS, n=70; LGS, n=121). Baseline characteristics are shown in **Table 1**. The most prevalent co-occurring conditions were psychomotor/cognitive impairment and sleep disorders (**Table 2**). At the time of the survey, over the prior 4 weeks moderate-to-very-severe physical

and mental impairments were reported in 65% (DS, 64%; LGS, 65%) and 77% (DS, 74%; LGS, 79%) of patients, respectively. Most patients received antiseizure medication (ASM) treatment before diagnosis of DS or LGS (overall, 89%; DS, 86%; LGS, 91%), with 32% having prior CBD exposure (DS, 35%; LGS, 30%). After DS or LGS diagnosis, CBD was the first ASM prescribed in 39% of patients (DS, 40%; LGS, 39%). The median [Q1, Q3] duration of CBD treatment among current users was 14.4 [6.4, 25.2] months (DS, 13.5 [5.6, 22.1]; LGS, 14.7 [7.2, 27.6]). Physicians reported satisfaction with current CBD-containing regimens for 87% of patients (DS, 87%; LGS, 88%). Top reasons for prescribing CBD included its effectiveness in reducing seizure severity (overall, 74%; DS, 82%; LGS, 68%) and seizure frequency (overall, 66%; DS, 69%; LGS, 63%).

Conclusions: In this multicountry study, CBD was prescribed to DS and LGS patients with seizures, a range of severity in physical and mental impairments, and various co-occurring conditions. Physicians reported satisfaction with current CBD-containing treatment regimens for the majority of patients with DS/LGS. These findings underscore the effectiveness of CBD in treating DS/LGS patients across all severity levels.

Funding: The DSP is an Adelphi Real World product, and all ownership of the data is retained. Jazz Pharmaceuticals was one of multiple subscribers to the Adelphi DS & LGS Disease Specific Programme dataset and funded this secondary analysis of the DSP.

Anti-seizure Medications

Table 1. Patient characteristics

	Overall (n=191)	DS (n=70)	LGS (n=121)
Median (Q1, Q3) age at time of survey, years	14.0 (6.0, 19.0)	10.0 (4.0, 17.0)	15.0 (8.0, 21.5)
Sex, n (%)			
Male	119 (62)	41 (59)	78 (64)
Female	72 (38)	29 (41)	43 (36)
Median (Q1, Q3) age at first seizure, years	2.0 (0.7, 4.0)	0.8 (0.6, 1.7)	3.0 (1.2, 5.0)
Median (Q1, Q3) age at diagnosis, years	4.1 (2.0, 6.5)	1.7 (0.8, 4.0)	5.2 (3.4, 8.0)
Median (Q1, Q3) time on current CBD treatment regimen, months	14.4 (6.4, 25.2)	13.5 (5.6, 22.1)	14.7 (7.2, 27.6)

DS, Dravet syndrome; LGS, Lennox-Gastaut syndrome; Q1, quartile 1; Q3, quartile 3.

Table 2. Diagnosis of comorbid conditions before or after time of DS/LGS diagnosis

		Overall n	DS n	LGS n
Psychomotor/cognitive impairment	Reported at diagnosis	53	23	30
	Reported after diagnosis	27	14	13
Sleep disorder/insomnia	Reported at diagnosis	30	13	17
	Reported after diagnosis	29	12	17
Autism	Reported at diagnosis	24	5	19
	Reported after diagnosis	27	13	14
Attention Deficit Hyperactivity Disorder	Reported at diagnosis	17	2	15
	Reported after diagnosis	31	19	12
Anxiety	Reported at diagnosis	13	3	10
	Reported after diagnosis	18	8	10

DS, Dravet syndrome; LGS, Lennox-Gastaut syndrome.