3.428 Seizure Outcomes With Cannabidiol in Pediatric Versus Adult Patients With Lennox-Gastaut Syndrome and Dravet Syndrome: Subgroup Analysis of BECOME, a Caregiver Survey

Timothy B Saurer,[1] Anne T Berg,[2] Tracy Dixon-Salazar,[3] Mary Anne Meskis,[4] Sherry R Danese,[5] Ngoc Minh D Le,[1] and M Scott Perry[6]

[1]Jazz Pharmaceuticals, Carlsbad, CA, USA; [2]Northwestern University Feinberg School of Medicine, Chicago, IL, USA; [3]Lennox-Gastaut Syndrome (LGS) Foundation, San Diego, CA, USA; [4]Dravet Syndrome Foundation, Cherry Hill, NJ, USA; [5]Outcomes Insights, Ventura, CA, USA; [6]Cook Children's Medical Center, Fort Worth, TX, USA

Background

- BECOME was a global outcomes survey of caregivers for people with Lennox-Gastaut syndrome (LGS) or Dravet syndrome (DS) that assessed changes in **BE**havior, **CO**gnition, and **M**ore with **E**pidiolex® (cannabidiol [CBD]).[1,2]
- In the primary analysis, a substantial proportion of caregivers reported improvements in seizure and nonseizure outcomes.[1,2]
  - Nearly all caregivers reported planning to continue CBD
- Although the onset of LGS and DS is usually in infancy or childhood, they are lifelong diseases with symptoms that evolve over time, emphasizing the need to understand the effects of CBD in pediatric vs adult patients.[3]
- We conducted a subgroup analysis to compare the seizure and nonseizure outcomes of Epidiolex treatment in pediatric (aged <18 years) and adult (aged ≥18 years) patients with LGS or DS.
  - This poster presents the seizure outcomes (nonseizure outcomes will be presented in Poster 3.429)

Objective

- To compare the seizure outcomes of Epidiolex treatment in pediatric vs adult patients with LGS or DS.

Methods

- US-based caregivers of patients with LGS or DS who received ≥3 months of CBD treatment (Epidiolex, 100 mg/mL oral solution) were asked to compare the month before survey administration with the period prior to CBD initiation.
- 'Don't Recall' or 'Not Applicable' responses were excluded. Net percentages included respondents' answers to ≥1 question within each domain.
- The survey consisted of multiple-choice and rank-order questions, based on validated measures and other previously published caregiver reports,[4–8] and used a symmetrical 3-, 5-, or 7-point Likert scale depending on the domain (from worsening to improvement).

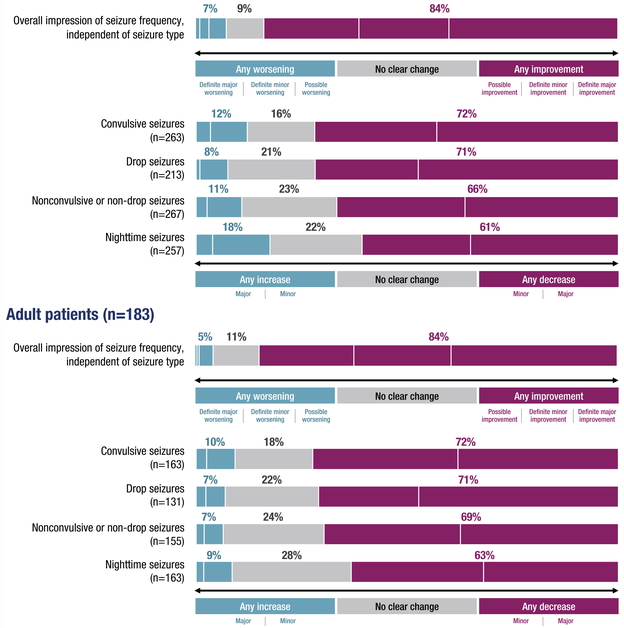
Results

Table 1. Patient characteristics and CBD exposure

	Pediatric patients (n=315)	Adult patients (n=183)	All patients (N=498)
Mean, y (range)	9 (1–17)	28 (18–73)	16 (1–73)
Male, n (%)	165 (52)	96 (53)	261 (52)
Responding caregivers, n (%)			
Parent	310 (98)	172 (94)	482 (97)
Grandparent	3 (1)	1 (1)	4 (1)
Other	2 (1)	10 (5)	12 (2)

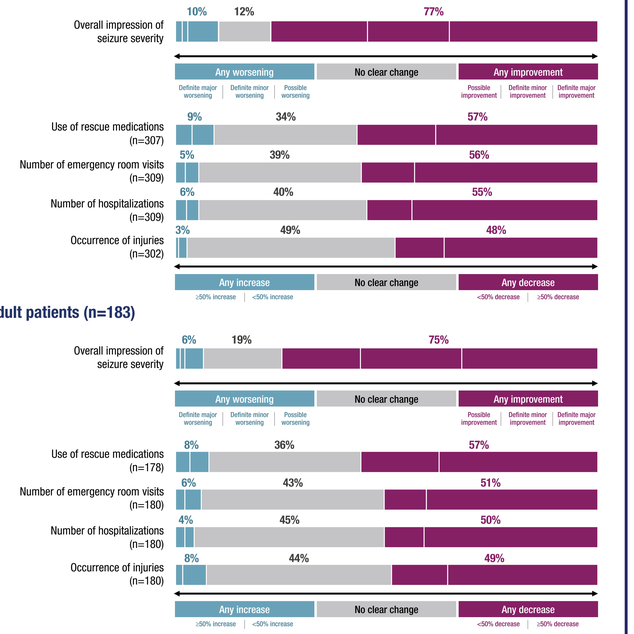
- Pediatric patients were treated with CBD for an average of 1.9 years and were taking a median (Q1, Q3) CBD dose of 16 mg/kg/d (9, 20).
- Adult patients were treated with CBD for an average of 2.2 years and were taking a median (Q1, Q3) CBD dose of 11 mg/kg/d (7, 17).
- Additional details for patients' concomitant antiseizure medications can be viewed via the QR code.

Figure 1. Seizure frequency



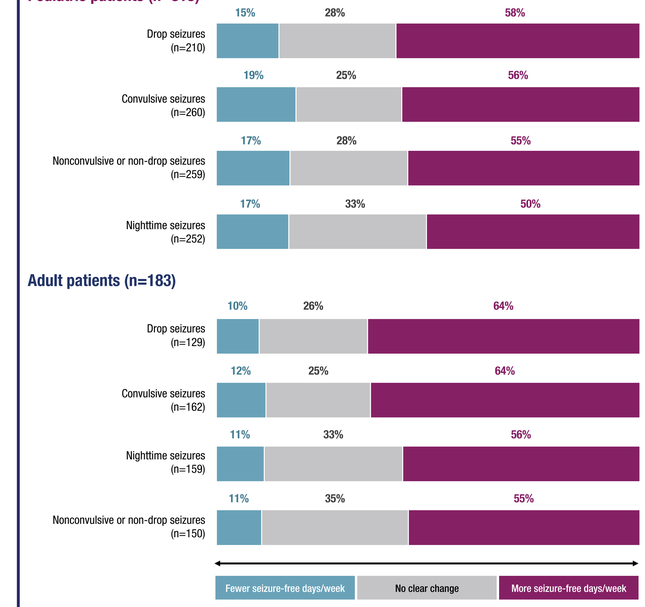
- Improvement in ≥1 type of seizure was reported by a net 84% of caregivers of pediatric and adult patients each, and worsening was reported by a net 21% of caregivers of pediatric patients and a net 19% of caregivers of adult patients.

Figure 2. Seizure severity



- Improvement in ≥1 question of the seizure severity domain was reported by a net 68% and 67% of caregivers of pediatric and adult patients, respectively; worsening was reported by a net 12% and 13% of caregivers of pediatric and adult patients.

Figure 3. Seizure-free days



- Increase in seizure-free days per week for ≥1 seizure type was reported by 65% of caregivers of pediatric patients and 70% of caregivers of adult patients.
- Seizure freedom in the past month was reported by 18% of caregivers of pediatric patients and 15% of caregivers of and adult patients.

Conclusions

- A substantial proportion of caregivers of patients with LGS or DS, regardless of age, reported improvements in patients' seizure frequency and severity and seizure-free days per week with CBD treatment.
- Caregivers for adult and pediatric patients reported similar improvements with CBD across the following seizure-related outcomes:
  - Seizure frequency independent of seizure type (84% in each group)
  - Seizure severity (77% vs 75% for the pediatric and adult patient groups, respectively)
  - Seizure-free days per week in ≥1 seizure type (65% vs 70%)
  - Complete seizure freedom in the past month (18% vs 15%)
- Improvements in nonseizure outcomes in pediatric vs adult patients were also reported (Poster # 3.429).
- Nearly all caregivers (93%) of pediatric and adult patients reported planning to continue CBD.
  - Although most caregivers of pediatric (93%) and adult (88%) patients cited seizure-related improvements as a reason for continuing, 79% of caregivers of pediatric patients and 73% of caregivers of adult patients cited nonseizure-related improvements as the reason (additional details available via the QR code and Poster # 3.429)

References: **1.** Dixon-Salazar T, et al. Presented at the AES Annual Meeting; December 3–7, 2021; Chicago, IL, USA. Abstract 3.3. **2.** Berg AT, et al. Presented at the AES Annual Meeting; December 3–7, 2021; Chicago, IL, USA. Abstract 3.304. **3.** Cross JH, et al. *Front Neurol.* 2017;8:505. **4.** Buck D, et al. *Epilepsy Behav.* 2007;10(1):38-43. **5.** Amtmann D, et al. *Qual Life Res.* 2020;29(5):1361-1371. **6.** Goodwin SW, et al. *Epilepsia.* 2018;59(3):668-678. **7.** Klatchoian DA, et al. *J Pediatr (Rio J).* 2008;84(4):308-315. **8.** Conway L, et al. *Epilepsia.* 2017;58(4):646-656.

Acknowledgments: Writing and editorial assistance were provided by Ritu Pathak, PhD, of Ashfield MedComms, an Inizio company, funded by Jazz Pharmaceuticals, Inc.

Support: BECOME was sponsored by Greenwich Biosciences, Inc (now part of Jazz Pharmaceuticals, Inc).

Disclosures: All authors met the ICMJE authorship criteria and had full access to relevant data. Neither honoraria nor payments were made for authorship. **ATB, TDS, MAM, SRD,** and **MSP** have consulted for, conducted studies funded by, or received honoraria from GW Pharmaceuticals companies (now part of Jazz Pharmaceuticals, Inc); **TBS** and **NMDL** were employees of Greenwich Biosciences, Inc (now part of Jazz Pharmaceuticals, Inc) at the time study was conducted; they are now employees of Jazz Pharmaceuticals, Inc. Epidiolex® is approved in the US for the treatment of seizures associated with Lennox-Gastaut syndrome, Dravet syndrome, or tuberous sclerosis complex in patients ≥1 years of age.

The American Epilepsy Society Annual Meeting 2022; December 2–6, 2022; Nashville, TN, USA

Scan this code to access this poster online. This code is not for promotional purposes.

3.428 Seizure Outcomes With Cannabidiol in Pediatric Versus Adult Patients With Lennox-Gastaut Syndrome and Dravet Syndrome: Subgroup Analysis of BECOME, a Caregiver Survey

Supplementary Material

Table S1. Patient characteristics and CBD exposure

	Pediatric patients (n=315)	Adult patients (n=183)	All patients (N=498)
Mean, y (range)	9 (1–17)	28 (18–73)	16 (1–73)
Male, n (%)	165 (52)	96 (53)	261 (52)
Number of concomitant ASMs, median (Q1, Q3)			
Current	4 (2, 4)	4 (3, 5)	4 (2, 5)
Most common ASMs in ≥20% of patients in any group, n (%)			
Clobazam	172 (55)	73 (40)	245 (49)
Clonazepam	83 (26)	44 (24)	127 (26)
Valproate	71 (23)	53 (29)	124 (25)
Levetiracetam	80 (25)	42 (23)	122 (25)
Lamotrigine	46 (15)	60 (33)	106 (21)
Responding caregivers, n (%)			
Parents	310 (98)	172 (94)	482 (97)
Grandparents	3 (1)	1 (1)	4 (1)
Other	2 (1)	10 (5)	12 (2)

ASM, antiseizure medication; CBD, cannabidiol; Q1, first quartile; Q3, third quartile.

Table S2. Caregivers' decision to continue CBD treatment

		Pediatric patients (n=315)	Adult patients (n=183)
		% of caregivers	
Do you plan to continue CBD for whom you are caring? (n=498)	Yes	93	93
	No	3	3
	Don't know	4	4
Of those who plan to continue (n=463), which are the most important reasons contributing to your decision to continue treatment?	Seizure related (net)	93	88
	Reduced seizure frequency	81	74
	Reduced seizure severity/duration	75	71
	Nonseizure related (net)	79	73
	Improved alertness	54	44
	Improved cognition	50	32
	Improved language/communication	33	24
	Improved sleep	33	25
	Improved physical functioning	31	18
	Improved social functioning	31	21
	Improved emotional functioning	32	25
	Reduced caregiver burden	19	15
	Other	5	11

CBD, cannabidiol.