

# Caregiver-Reported Nonseizure Outcomes With Real-World Use of Cannabidiol in Tuberous Sclerosis Complex: Interim Results From the BECOME-TSC Survey

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## Background

- Tuberous sclerosis complex (TSC) is a neurocutaneous disorder, characterized by the formation of hamartomas in multiple organs, including the brain, skin, heart, eyes, kidneys, lungs, and liver.<sup>1,2</sup>
- More than 90% of people with TSC have TSC-associated neuropsychiatric disorders (TAND), characterized by behavioral, psychiatric, intellectual, academic, neuropsychologic, and psychosocial problems.<sup>3-5</sup>
- The plant-derived, highly purified pharmaceutical formulation of cannabidiol (CBD) is approved in the United States (US) for the treatment of seizures associated with Lennox-Gastaut syndrome, Dravet syndrome, and TSC in patients aged ≥1 year.<sup>6</sup>
- BECOME-TSC (**BE**havior, **CO**gnition, and **MO**re with **E**pidiolex<sup>®</sup> in TSC) is an ongoing cross-sectional survey to quantify the real-world impact of CBD on seizure and nonseizure outcomes in people with TSC.
  - This poster presents the nonseizure outcomes (seizure outcomes will be presented separately).

## Objective

- To present caregiver-reported nonseizure outcomes following initiation of CBD treatment in people with TSC.

## Methods

- Using electronic health records, healthcare providers at TSC centers in the US identified people with TSC who were treated with CBD (Epidiolex<sup>®</sup>, 100 mg/mL oral solution) for ≥6 months.
- Caregivers of these people completed an online survey, consisting of multiple choice and rank order questions, based on the TAND questionnaire,<sup>7</sup> other validated measures, and previous caregiver reports.
- Respondents compared the past month to the period before CBD initiation and rated their impression of change using a symmetrical 3-, 5-, or 7-point Likert scale (from worsening to improvement) depending on the domain.
- 'Don't Recall' or 'Not Applicable' responses were excluded.
- Continuous variables were summarized as means, medians, and ranges, and categorical variables as frequency distributions and percentages.
- CBD-associated adverse events, which can include transaminase elevations, somnolence, decreased appetite, diarrhea, pyrexia, vomiting, fatigue, rash, sleep disorders, and infections, were not assessed.
- The survey was conducted with caregivers of people taking Epidiolex<sup>®</sup>, and the results do not apply to other CBD-containing products.

## Results

- At the time of analysis, 12 caregivers had completed the survey.

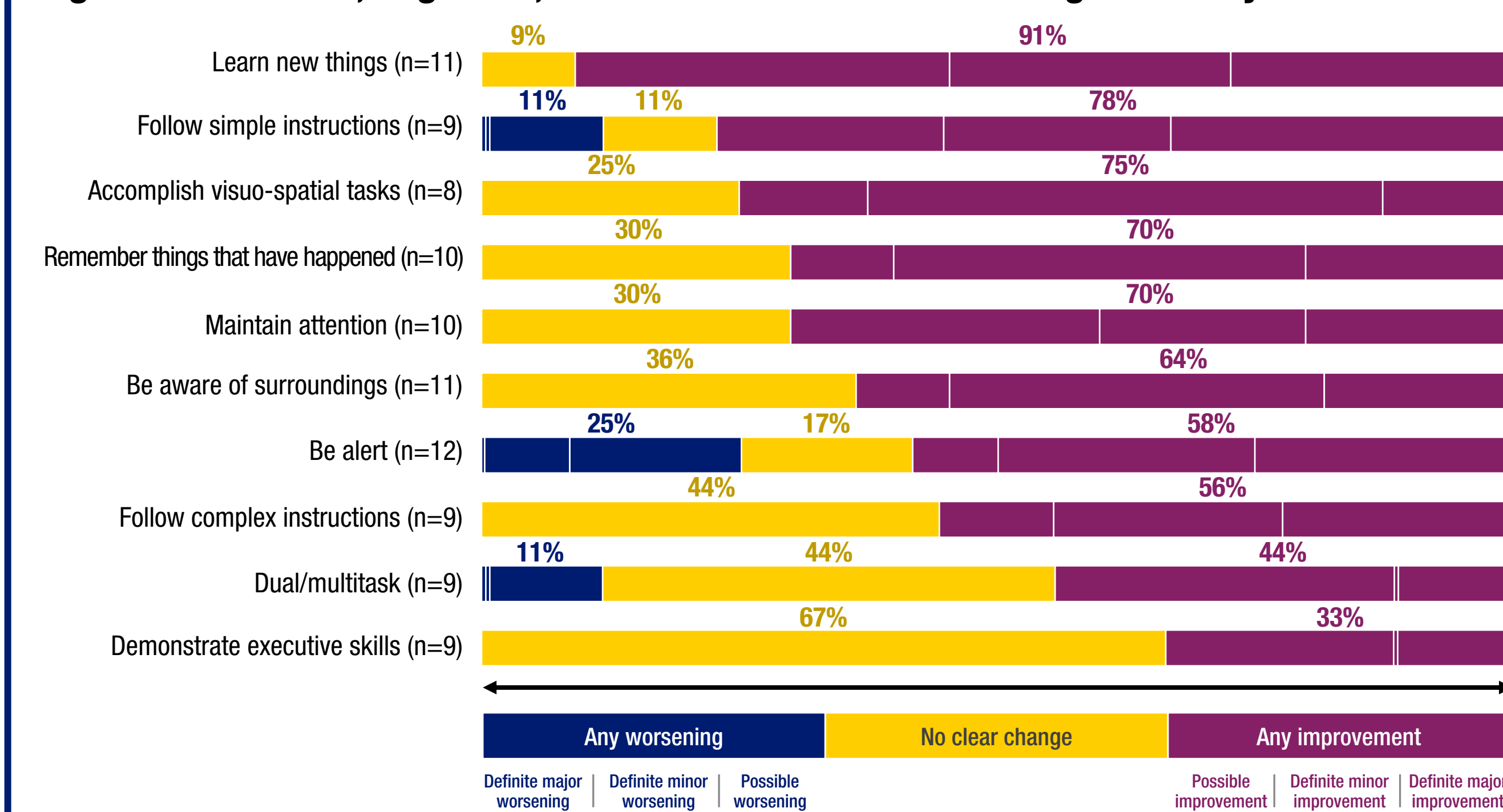
**Table 1. Characteristics of patients in the survey**

	Patients (N=12)
Mean age, years (SD)	16.2 (8.4)
Number of ASMs before CBD initiation, median (Q1, Q3)	4 (2, 5)
Most common concomitant (≥30%) ASMs, n (%)	
Everolimus	5 (42)
Clonazepam	4 (33)
Most common co-occurring conditions, n (%)	
Developmental delay	11 (92)
Autism spectrum disorder	10 (83)
Anxiety disorder	5 (42)
Intellectual disability, n (%)	
Severe-profound	9 (75)
Median CBD dose at the time of survey, mg/kg/d (Q1, Q3)	17 (15, 23)

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

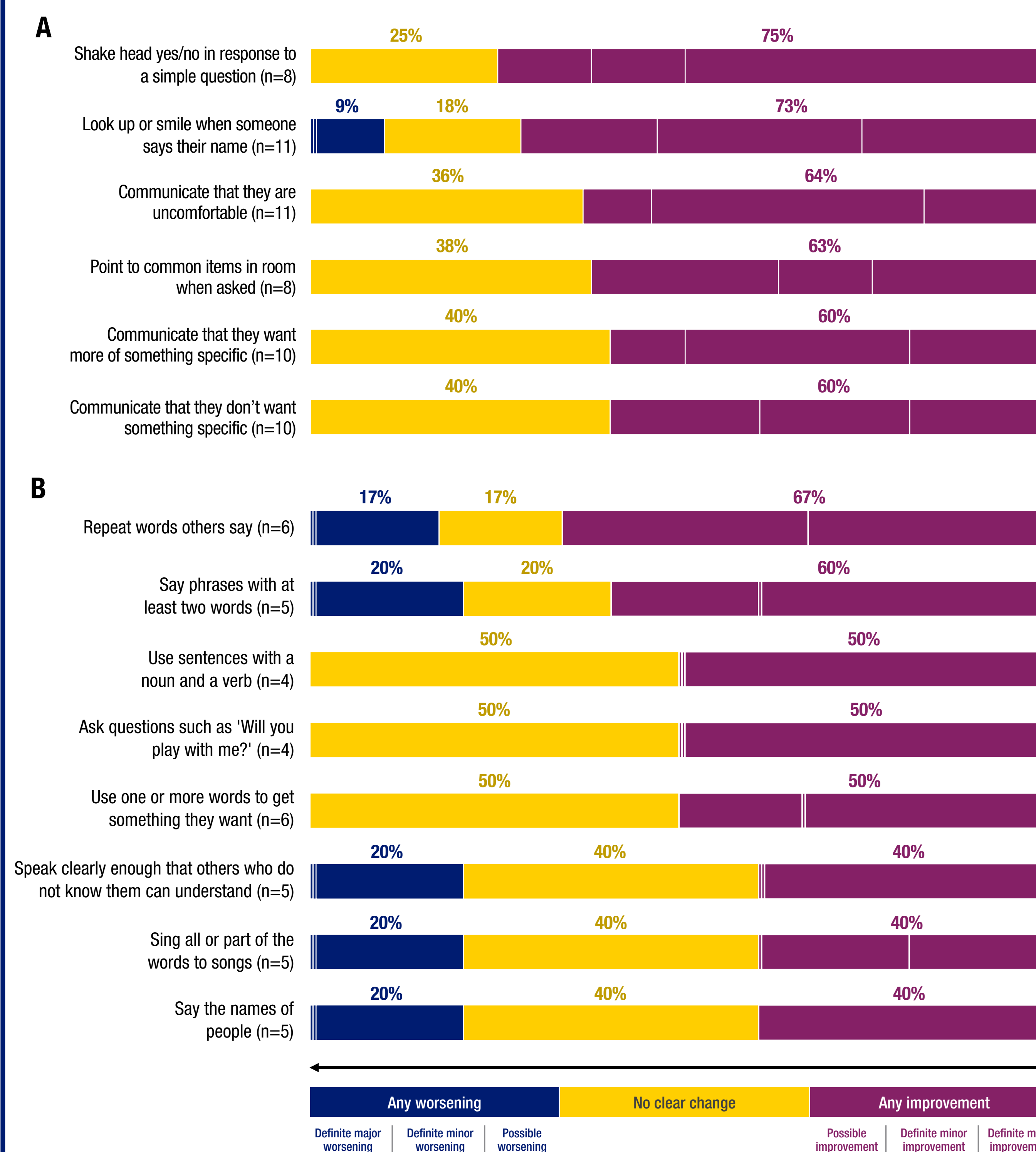
- Fifty percent of patients in the survey had a history of infantile spasms.

**Figure 1. Alertness, cognition, and executive function – change in ability to**



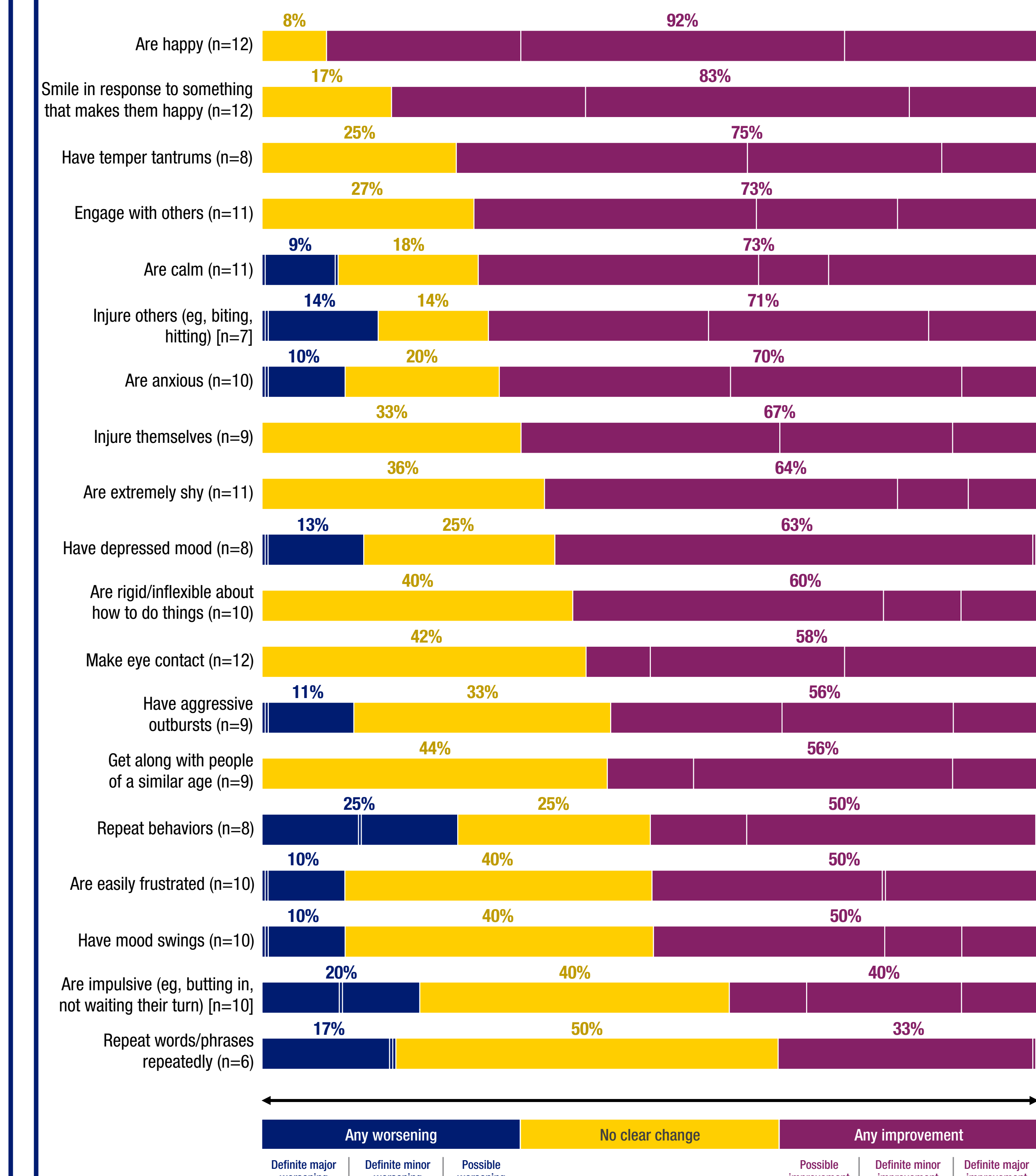
- At least 75% of the respondents reported improvements in the patient's ability to learn new things, follow simple instructions, and accomplish visuo-spatial tasks in the alertness, cognition, and executive function domain.

**Figure 2. Language and communication in (A) all patients and (B) patients with verbal abilities (few/minimal words or fluent verbal language) – change in ability to**



- The most frequently reported improvements in communication domain were in the ability to respond to a simple question (75%) in all patients and repeating words others say in verbal patients (67%).

**Figure 3. Emotional and social functioning – change in how often they**



- The most frequently reported improvement in emotional and social functioning was how often patients were happy (92%).

## Conclusions

- In this preliminary analysis of the ongoing BECOME-TSC survey:
  - Most caregivers reported improvements in the cognition, emotional functioning, and communication domains.
  - A total of 92% of caregivers reported planning to continue CBD and gave reduced seizure frequency, reduced seizure severity/duration (seizure outcomes Poster 2.503), and improved cognition as the most common reasons for continuation.
  - Limitations of the study include retrospective caregiver accounts and selection bias due to study design as well as a small sample size in this preliminary analysis. Adverse effects were not assessed and the effect of concomitant antiseizure medications was not considered in this analysis.
  - Most caregivers of people with TSC reported improvement in TAND-related nonseizure outcomes since initiating CBD.

**References:** 1. Northrup H et al. *Pediatr Neurol*. 2021;123:50-66. 2. Curatolo P et al. *Lancet*. 2008;372:657-668. 3. Vancooster S et al. *J Neurodevel Disord*. 2022;14:13. 4. Zöllner JP et al. *Orphanet J Rare Dis*. 2020;15:23. 5. de Vries PJ et al. *J Neurodevel Disord*. 2023;15:32. 6. Jazz Pharmaceuticals. Epidiolex<sup>®</sup> (cannabidiol) oral solution [prescribing information]. 2023. [https://www.epidiolex.com/sites/default/files/pdfs/1120/EPX-03645-1120\\_EPIDIOLEX\\_\(cannabidiol\)\\_USPI.pdf](https://www.epidiolex.com/sites/default/files/pdfs/1120/EPX-03645-1120_EPIDIOLEX_(cannabidiol)_USPI.pdf). 7. de Vries PJ et al. *Pediatr Neurol*. 2015;52:25-35.

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**Disclosures:** All authors met the ICMJE authorship criteria and had full access to relevant data. Neither honoraria nor payments were made for authorship. **SMW, MKK, DS, DAK, SM, CK, SRD** have consulted for, conducted studies funded by, or received honoraria for services provided to Jazz Pharmaceuticals, Inc; **TBS, KCS, and KR** are employees of Jazz Pharmaceuticals, Inc. Epidiolex<sup>®</sup> 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.



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