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.1, 2 More than 90% of people with TSC have TSC-associated neurocutaneous disorders (TANO), characterized by behavioral, psychiatric, intellectual, academic, neuropsychiatric, and psychosocial problems.3-6 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.7

BECOME TSC (BDeriv, CoPion, and Mend with Epidiolex® 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.

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®, 100 mg/mL, oral solution) for ≥6 months.

Caregivers of people who completed an online survey, consisting of multiple choice and rank order questions, based on the TANO questionnaire,1 other validated measures, and previous caregiver reports. Respondents compare the past month to the period before CBD initiation and rated their impression of change using a symptom-specific, 5-, 7- or 9-point Likert scale (from worsening to improvement) depending on the domain.

"Don’t Recall" or "Not Applicable" responses were excluded.

The survey was conducted with caregivers of people taking Epidiolex® for ≥6 months. Results from the BECOME-TSC survey are presented for those with ≥6 months of data available.

The survey included caregivers from TSC centers in the United States, Canada, and the United Kingdom. Caregivers were recruited from patient support groups, patient registries, and TSC conference presentations.

Results

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

Table 1. Characteristics of patients in the survey

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#### Background

The University of Texas Health Science Center at Houston, Houston, TX, USA; – and the results do not apply to other CBD-containing products.

#### Results

- Most respondents reported improvements in the patient’s ability to learn new things, follow simple instructions, and complete visuospatial tasks in the alertness, cognition, and executive function domain.

- The most frequently reported improvements in communicative domain were in the ability to respond to a simple question (36%), in patients and repeating words others say in verbal patients (67%).

#### Conclusions

- Most caregivers reported improvements in the cognitive, emotional functioning, and communication domains.

- A total of 90% of caregivers reported planning to continue CBD and gave reduced seizure frequency, reduced seizure severity/duration, 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 TANO-related nonseizure outcomes following initiating CBD.

#### Footnotes


#### Acknowledgments

The authors acknowledge the contribution of all caregivers who participated in the BECOME TSC study.

#### Support

The study was funded by Takeda Pharmaceuticals International Inc. The study was conducted in accordance with all relevant and approved guidelines for the conduct of clinical studies.