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.4-7 Epilepsy is the most prevalent neurological manifestation of TSC, with seizures often starting in infancy and may persist evolving with multiple seizure types.8,9 Treatment-resistant seizures associated with TSC are a significant and frequent cause of morbidity in people with TSC.1,4 The plant-derived, highly purified pharmaceutical formulation of cannabidiol (CBD) is approved in the United States for the treatment of seizures associated with Lennox-Gastaut syndrome, Dravet syndrome, and TSC in people aged 2 years and older.10-12 BECOME-TSC (Bellevue, Oigdon, and Mir) with Epidiolex® in a cross-sectional survey to quantify the real-world impact of CBD on seizure and nonseizure outcomes in people with TSC. This poster presents the seizure outcomes (nonseizure outcomes will be presented separately).

Objective

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

Methods

Using electronic health records, healthcare professionals at TSC centers in the US identified people with TSC who were treated with CBD Epidiolex®, 100 mg/mL oral solution for 12 months. Caregivers of these individuals completed an online survey, consisting of multiple choice and rank-order questions, based on the TSC-Associated Neuropsychiatric Disorders questionnaire, other validated scales, and previous caregiver reports.

Respondents compared the past month to the period before CBD initiation and rated their impression of change using a symmetrical 1-5; 1=very bad/10=very good scale (from worsening to improvement) depending on the domain.

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

Continuous variables were summarized as mean, medians, and ranges, and categorical variables as frequency distributions and proportions.

CBD-associated adverse events, which can include transaminase elevations, somnolence, decreased appetite, diarrhea, pyrexia, inattentive (n=9).

Results

More than 50% of respondents reported improvements in the frequency of seizures that involve movement or stiffening of both or one side of the body, nighttime seizures, status epilepticus, and seizures where one is less responsive/inattentive.

More than 30% of respondents reported decrease in seizure medication use (38%) and occurrence of seizures-related injuries (50%).

Conclusions

In this preliminary analysis of BECOME-TSC, an ongoing cross-sectional survey of caregivers of people with TSC who are taking CBD treatment.

• Most caregivers reported improvements in seizure frequency (83%) and severity (92%).

• Complete seizure freedom in the past month was reported by 42% of respondents.

• Improvements were most commonly reported in the frequency of seizures that involve movement or stiffening of both or one side of the body, nighttime seizures, status epilepticus, and seizures where one is less responsive/inattentive.

• The majority of respondents planned to continue CBD treatment primarily because of reduced seizure severity/duration but also because of improvements in nonseizure outcomes, including cognition, emotional function, and language/communication.

• Limitations of the study include small sample size, use of retrospective caregiver accounts, and selection bias because of the study design. Adverse effects were not assessed and the effect of concomitant antiseizure medications was not considered in this analysis.

References


Acknowledgments

The study was conducted with caregivers of people taking Epidiolex®, and the results do not apply to other CBD-containing products.

Editorial assistance in the development of this manuscript was provided by the White, B.P., and Bonn-Bryson of United Medical, health company, Jazz Pharmaceuticals. Support for medical writing was provided by Jazz Pharmaceuticals, Inc.

Disclosure: The author or a member of the author’s immediate family has a financial interest in a drug or device described in this manuscript. Neither of the companies was involved in the design or conduct of this study, or the collection, analysis, or interpretation of the data. The author and the company were not involved in the preparation, review, or approval of the manuscript. The author and the company were also not involved in the decision to submit the manuscript for publication.

Neuroscience Nursing Annual Conference; March 4-19, 2018; Salt Lake City, UT, USA.