



CADTH REIMBURSEMENT REVIEW

Patient and Clinician Group Input

cannabidiol (Epidiolex)
(Jazz Pharmaceuticals Canada, Inc.)

Indication: As adjunctive therapy for seizures associated with DS in patients two years of age and older.

October 10, 2023

This document compiles the input submitted by patient groups and clinician groups for the file under review. The information is used by CADTH in all phases of the review, including the appraisal of evidence and interpretation of the results. The input submitted for each review is also included in the briefing materials that are sent to expert committee members prior to committee meetings.

Disclaimer: The views expressed in this submission are those of the submitting organization or individual. As such, they are independent of CADTH and do not necessarily represent or reflect the views of CADTH. No endorsement by CADTH is intended or should be inferred.

By filing with CADTH, the submitting organization or individual agrees to the full disclosure of the information. CADTH does not edit the content of the submissions received.

CADTH does use reasonable care to prevent disclosure of personal information in posted material; however, it is ultimately the submitter's responsibility to ensure no identifying personal information or personal health information is included in the submission. The name of the submitting group and all conflicts of interest information from individuals who contributed to the content are included in the posted submission.

Patient Group Input

Patient Input Template for CADTH Reimbursement Reviews

1. About Your Patient Group

Describe the purpose of your organization. Include a link to your website.

The Canadian Epilepsy Alliance (CEA) is a Canada-wide network of grassroots organizations dedicated to the promotion of independence and quality of life for people with epilepsy and their families, through support services, information, advocacy, and public awareness. www.canadianepilepsyalliance.org

2. Information Gathering

CADTH is interested in hearing from a wide range of patients and caregivers in this patient input submission. Describe how you gathered the perspectives: for example, by interviews, focus groups, or survey; personal experience; or a combination of these. Where possible, include **when** the data were gathered; if data were gathered **in Canada** or elsewhere; demographics of the respondents; and **how many** patients, caregivers, and individuals with experience with the drug in review contributed insights. We will use this background to better understand the context of the perspectives shared.

Information for this submission has been gathered by the President of the Canadian Epilepsy Alliance, drawing on the knowledge and experiences of its 24 member associations, and the lived experiences and firsthand knowledge of: patients, caregivers, clinicians, volunteers, and supporters (donors/funders) through the decades of experience and the work that each of the member associations performs in communities across Canada.

3. Disease Experience

CADTH involves clinical experts in every review to explain disease progression and treatment goals. Here we are interested in understanding the illness from a patient's perspective. Describe how the disease impacts patients' and caregivers' day-to-day life and quality of life. Are there any aspects of the illness that are more important to control than others?

Current therapies work for nearly 70% of persons with Epilepsy. The remaining 30% try to remain hopeful that someday a medication will be found that will help them. New drugs like Cannabidiol bring hope to many who are close to giving up. When epilepsy is not controlled by medications, the impact of the disease on individuals and their families is significant. Individuals with uncontrolled epilepsy can be socially isolated due to stigma due to fear of rejection in social, work, and educational situations. There is a high correlation of mental illness such as depression and anxiety that accompany and initial diagnosis and these can linger when anti-seizure medications do not provide relief.

When someone has epilepsy, the whole family is affected. Everyone's life revolves around the seizures. There is anxiety around when and where the next seizure will occur, and what impact it will have. A husband is afraid his wife might have a seizure and drop the baby; parents are nervous if their child is invited to a birthday party; a teenager is anxious as he watches his father leave for work. Is today the day that something terrible will happen?

Some caregivers are afraid to leave the person with frequent seizures alone, contributing to a loss of independence and the lack of self-esteem we see so often with this patient population. Compassion fatigue in the care giver is always of concern. Many caregivers are sleep deprived as they either try to stay awake all night in case a seizure happens or go to bed and find they are too anxious to sleep.

As well, caregivers often have to live with the sometimes highly unpleasant side effects of various medications that their loved ones are taking. Mood swings, sexual dysfunction, suicidal thoughts, memory loss, problems with concentration, fatigue, exhaustion, depression – all can prove devastating to the person involved, and also to those around them.

4. Experiences With Currently Available Treatments

CADTH examines the clinical benefit and cost-effectiveness of new drugs compared with currently available treatments. We can use this information to evaluate how well the drug under review might address gaps if current therapies fall short for patients and caregivers.

Describe how well patients and caregivers are managing their illnesses with currently available treatments (please specify treatments). Consider benefits seen, and side effects experienced and their management. Also consider any difficulties accessing treatment (cost, travel to clinic, time off work) and receiving treatment (swallowing pills, infusion lines).

No drug works for everyone, and each new drug brought to market offers hope to the 30% of epilepsy sufferers whose seizures are uncontrolled by one or a combination of existing therapies. Even a reduction in the absolute number of seizures that these individuals experience can potentially improve overall quality of life. Seizure freedom may become a reality. Patients without seizure control are always hopeful that a new therapy lies around the corner and without access to safe, approved therapies, some begin to experiment with alternative medicines or practices (cannabis and other unregulated substances). This experimentation can ultimately prove not only detrimental, but also hazardous to the health of those affected.

5. Improved Outcomes

CADTH is interested in patients' views on what outcomes we should consider when evaluating new therapies. What improvements would patients and caregivers like to see in a new treatment that is not achieved in currently available treatments? How might daily life and quality of life for patients, caregivers, and families be different if the new treatment provided those desired improvements? What trade-offs do patients, families, and caregivers consider when choosing therapy?

People with intractable epilepsy are very often unemployed or under-employed because of the frequency of their seizures. They usually live on very restricted incomes, and because they are not working, or are only working parttime, the majority is without access to employer-funded insurance plans. If new medications are not placed on the Provincial formulary, the majority of those with intractable epilepsy, the ones who need them the most, will never be given the opportunity to find out if this new drug will work for them.

6. Experience With Drug Under Review

CADTH will carefully review the relevant scientific literature and clinical studies. We would like to hear from patients about their individual experiences with the new drug. This can help reviewers better understand how the drug under review meets the needs and preferences of patients, caregivers, and families.

How did patients have access to the drug under review (for example, clinical trials, private insurance)? Compared to any previous therapies patients have used, what were the benefits experienced? What were the disadvantages? How did the benefits and disadvantages impact the lives of patients, caregivers, and families? Consider side effects and if they were tolerated or how they were managed. Was the drug easier to use than previous therapies? If so, how? Are there subgroups of patients within this disease state for whom this drug is particularly helpful? In what ways? If applicable, please provide the sequencing of therapies that patients would have used prior to and after in relation to the new drug under review. Please also include a summary statement of the key values that are important to patients and caregivers with respect to the drug under review.

These are questions we cannot answer at this time. We support the concept of a new drug that, through the results of trials, offers hope - that in itself offers improvement in an individual's overall outlook, health and well-being.

7. Companion Diagnostic Test

If the drug in review has a companion diagnostic, please comment. Companion diagnostics are laboratory tests that provide information essential for the safe and effective use of particular therapeutic drugs. They work by detecting specific biomarkers that predict more favourable responses to certain drugs. In practice, companion diagnostics can identify patients who are likely to benefit or experience harms from particular therapies or monitor clinical responses to optimally guide treatment adjustments.

What are patient and caregiver experiences with the biomarker testing (companion diagnostic) associated with regarding the drug under review?

Consider:

- Access to testing: for example, proximity to testing facility, availability of appointment.
- Testing: for example, how was the test done? Did testing delay the treatment from beginning? Were there any adverse effects associated with testing?

- Cost of testing: Who paid for testing? If the cost was out of pocket, what was the impact of having to pay? Were there travel costs involved?
- How patients and caregivers feel about testing: for example, understanding why the test happened, coping with anxiety while waiting for the test result, uncertainty about making a decision given the test result.

The Canadian Epilepsy Alliance does not have sufficient data to comment on this at this time.

8. Anything Else?

Is there anything else specifically related to this drug review that CADTH reviewers or the expert committee should know?

No

Appendix: Patient Group Conflict of Interest Declaration

To maintain the objectivity and credibility of the CADTH reimbursement review process, all participants in the drug review processes must disclose any real, potential, or perceived conflicts of interest. This Patient Group Conflict of Interest Declaration is required for participation. Declarations made do not negate or preclude the use of the patient group input. CADTH may contact your group with further questions, as needed.

1. Did you receive help from outside your patient group to complete this submission? If yes, please detail the help and who provided it.

<Enter Response Here> N/A

2. Did you receive help from outside your patient group to collect or analyze data used in this submission? If yes, please detail the help and who provided it.

<Enter Response Here>N/A

3. List any companies or organizations that have provided your group with financial payment over the past 2 years AND who may have direct or indirect interest in the drug under review.

Financial Disclosures

Check Appropriate Dollar Range With an X. Add additional rows if necessary.

Company	\$0 to 5,000	\$5,001 to 10,000	\$10,001 to 50,000	In Excess of \$50,000
<Enter Name Here>				

I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this patient group with a company, organization, or entity that may place this patient group in a real, potential, or perceived conflict of interest situation.

Name: Laura Dickson

Position: President

Patient Group: Canadian Epilepsy Alliance

September 29, 2023

Clinician Group Input

No clinician group input was received by CADTH.