6.16 CANNABIDIOL, Oral liquid 100 mg per mL, 100 mL, Epidyolex®, Jazz Pharmaceuticals ANZ Pty Ltd

1 Purpose of Submission

1.1 The Category 3 submission requested a change to the restriction level of PBS-listed cannabidiol (Epidyolex®) for the treatment of seizures associated with Dravet syndrome (DS), from Authority Required (Telephone/online PBS Authorities system) to Authority Required (STREAMLINED).

1.2 The submission also requested amendments to the treatment criteria to allow prescribing by a paediatrician without the need for consultation with a neurologist, and continuation of therapy by a general practitioner in consultation with a paediatrician.

2 Background

2.1 Cannabidiol is currently listed on the PBS as a General Schedule Authority Required (Telephone/online PBS Authorities system) listing for severe myoclonic epilepsy in infancy (Dravet syndrome (DS)).

2.2 Cannabidiol is also listed on the PBS as an Authority Required (STREAMLINED) benefit for seizures of the Lennox-Gastaut syndrome (LGS).

Registration status

2.3 Cannabidiol was TGA registered on 21 September 2020 for use as adjunctive therapy of seizures associated with LGS, or Dravet syndrome for patients 2 years of age or older. The TGA approved product information (PI) indicates that cannabidiol should be initiated and supervised by a neurologist.

Previous PBAC consideration

2.4 At its PBAC meeting November 2020, the PBAC recommended the listing of cannabidiol for the treatment of DS in combination with at least two other anti-epileptic drugs (AEDs) on the PBS. The PBAC considered cannabidiol would be of acceptable cost-effectiveness for DS if it were listed at a cost per patient per year that is less than, or is not significantly higher than, that for stiripentol.

2.5 The PBAC has not previously considered amending the restriction level from Authority Required (Telephone/online PBS Authorities system) to Authority Required (STREAMLINED) and amending the treatment criteria to allow prescribing by a paediatrician without the need for consultation with a neurologist for DS.

3 Requested listing

3.1 The submission requested the following changes to the existing listing (PBS item code: 12467E), as outlined below. The submission's proposed additions are shown with bold text, and deletions are indicated with strikethrough with regular text. Additions proposed by the Secretariat are shown in italics and Secretariat-proposed deletions are shown in italics and strikethrough.

Amend existing listing as follows:

MEDICINAL PRODUCT **medicinal product pack**		**PBS item code**	**Max. qty packs**	**Max. qty units**	**№.of Rpts**	**Available brands**
CANNABIDIOL						
cannabidiol 100 mg/mL oral liquid, 100 mL		12467E	1	1	5	Epidyolex
Restriction Summary 17572/ Treatment of Concept: 17521						
Concept ID (for internal Dept. use)	**Category / Program:** ☒ GENERAL - General Schedule (Code GE)					
	Prescriber type: ☒Medical Practitioners					
	Restriction type: ☒***Authority Required (Streamlined)*** ☒~~Authority Required (telephone/online PBS Authorities system)~~					
Prescribing rule level	~~**Administrative Advice:** *Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 888 333.*~~					
	Administrative Advice: Requests for increased quantities may be sought based on daily doses not exceeding 20 mg/kg/day (in line with the Product Information) for up to 4 weeks per dispensing.					
	Administrative Advice: No increase in the maximum number of repeats may be authorised.					
	Administrative Advice: Special Pricing Arrangements apply.					
	Indication: Severe myoclonic epilepsy in infancy (Dravet syndrome)					
	Clinical criteria:					
	Patient must have (as an initiating patient)/have had (as a continuing patient), generalised tonic-clonic seizures or generalised clonic seizures that are not adequately controlled with at least two other antiseizure medications					
	AND					
	Clinical criteria:					
	The treatment must be as adjunctive therapy to ~~*at least two*~~ other antiseizure medication~~s~~					
	Treatment criteria					
	Must be treated by a *prescriber who is either: (i) a* neurologist ~~**or**~~ *(ii)* **paediatrician** if treatment is being initiated; or					
	Must be treated by a *prescriber who is either: (i) a* neurologist ~~**or**~~ *(ii)* **paediatrician** if treatment is being continued or re-initiated; or					
	~~Must be treated by a paediatrician in consultation with a neurologist if treatment is being continued; or~~					
	Must be treated by a general practitioner in consultation with *either: (i)* a neurologist (ii) ~~**or**~~ **paediatrician** if treatment is being continued					
	Prescribing Instructions: Confirmation of eligibility for treatment with diagnostic reports must be documented in the patient's medical records.					

3.2 The Secretariat noted that the proposed changes to the restriction criteria were not consistent with the TGA-approved PI, which specifies the use of cannabidiol as

adjunctive therapy in patients with DS, to be initiated and supervised by a neurologist. The proposed amendments would allow initiation and continuation by a paediatrician without consultation with a neurologist and enable continued use of cannabidiol without the requirement for concomitant anti-epileptic drugs (AEDs). In the criterion: "the treatment must be as adjunctive therapy to at least two other antiseizure medications ", the Secretariat proposed removing "at least two" to ensure cannabidiol use as an add-on therapy with any number of concomitant AEDs, consistent with TGA registration, and clinical practice.

3.3 In July 2025 PBAC meeting, the PBAC recommended the following changes to the restrictions of cannabidiol for the treatment of seizures associated with LGS:

- amending the restriction level of cannabidiol for the treatment of seizures associated with LGS, from Authority Required (Telephone/online PBS Authorities system) to Authority Required (STREAMLINED).
- Remove the requirement for "at least two" other antiseizure medication, allowing ongoing cannabidiol use with one or more concomitant AEDs.
- Amend the treatment criteria to allow prescribing by a paediatrician without the need for consultation with a neurologist, and continuation of therapy by a general practitioner in consultation with a paediatrician.

3.4 At the July 2025 meeting, the PBAC noted that utilisation of cannabidiol was lower than anticipated, that prescribers now have considerable experience with its use, and that the risk of prescribing to ineligible patients is low. Therefore, the PBAC considered a streamlined authority to be appropriate.

3.5 The proposed change to the restriction level and treatment criteria of Epidyolex for the treatment of DS is consistent with the PBAC's recommendation for Epidyolex for the treatment of LGS (PBS item number 13277T) at its July 2025 PBAC meeting.

4 Consideration of the evidence

Sponsor hearing

4.1 There was no hearing for this item.

Consumer input

4.2 The PBAC noted and welcomed input received via the Office of Health Technology Assessment Consultation Hub from neurologists (n=4), a medical organisation (n=1), and consumer groups (n=2). All inputs supported the proposed changes to the restriction level of PBS-listed cannabidiol for the treatment of seizures associated with DS.

4.3 Neurologists expressed support for the proposed amendments to the cannabidiol listing. Inputs highlighted the effectiveness and safety of cannabidiol in the treatment

of DS, noting its potential to improve patients' quality of life. Commenters further noted that changing the listing from Authority Required to Authority Required (Streamlined) would improve access to cannabidiol and enhance the efficiency of the prescribing process.

4.4 Epilepsy Action Australia, the Epilepsy Foundation, and the Pharmaceutical Society of Australia provided submissions in support of amending the PBS restrictions for cannabidiol. This included lowering the restriction level to Authority Required (Streamlined) and amending the clinical criteria to allow paediatricians to initiate treatment to allow a general practitioner to continue therapy in consultation with a paediatrician. The submissions described that these changes would improve equity of access and reduce delays in treatment initiation.

Clinical trials

4.5 The submission did not present any new clinical evidence in support of the proposed amendments to the current restriction criteria.

Rationale for the proposed changes

4.6 The submission stated that specialist physicians have advocated for a streamlined authority process for Epidyolex for the treatment of seizures associated with DS. The submission also stated that epilepsy specialist physicians have reported to the sponsor that current PBS authority process demands 10-20 minutes of their time per patient to call or complete. The submission stated that a streamlined authority process for DS would help reduce the administrative burden on an already under-resourced specialty on prescribers, as they do not need to seek prior telephone or written approval.

4.7 The submission also argued that Australia currently has a shortage of specialty physicians with subspecialty qualifications in epileptology, with an associated backlog of patients requiring specialist management. The submission stated that the proposed Streamlined authority will encourage greater involvement of GPs in the long-term management of patients with DS, and ultimately supporting improved patient care.

4.8 The submission stated that consultation with epilepsy specialist physicians has highlighted that the current restriction criteria contribute to inequitable access to cannabidiol, particularly for adult patients and those living in rural and remote areas where there are no paediatric neurologists.

4.9 The submission stated that healthcare professionals understand Epidyolex is not a drug of misuse/abuse and contains no tetrahydrocannabinol.

Economic analysis

4.10 No economic analysis was provided, as the submission stated that the proposed changes to the PBS restrictions would not impact the cost-effectiveness of cannabidiol. The PBAC considered this was appropriate as eligible patient population would likely remain the same.

Estimated PBS usage and financial implications

4.11 The submission assumed there would be no financial impact to the PBS/RPBS from this change to the authority level (i.e. no expected change to the patient population, current treatment algorithm or price per unit). Based on the available information, the proposed change may affect the number of Authority Required services processed by Services Australia. The financial impact to Services Australia will be determined by that agency as part of the post PBAC process.

4.12 The Pre-PBAC response indicated that the actual PBS prescription volume for cannabidiol in the DS indication over the first five years since its listing in May 2021 (5,000 to < 10,000 scripts) has been slightly higher than the originally estimated volume of 5,000 to < 10,000 scripts provided in the November 2020 PBAC submission. The PBAC noted that there was a small population that benefits from this treatment. The PBAC advised that the estimated nil net financial impact to the PBS/RPBS was appropriate.

5 PBAC Outcome

5.1 The PBAC recommended amending the authority requirements for cannabidiol for the treatment of seizures associated with Dravet syndrome (DS), from Authority Required (Telephone/online PBS Authorities system) to Authority Required (STREAMLINED).

5.2 The PBAC also recommended the following amendments to the current PBS criteria for cannabidiol in the DS indication:

- Remove the requirement for “at least two” other antiseizure medication, allowing ongoing cannabidiol use with one or more concomitant AEDs.
- Amend the treatment criteria to allow prescribing by a paediatrician without the need for consultation with a neurologist, and continuation of therapy by a general practitioner in consultation with a paediatrician;

5.3 The PBAC noted and welcomed input from neurologists, Epilepsy Action Australia, Epilepsy Foundation and Pharmaceutical Society of Australia. The PBAC noted that all inputs described that the recommended amendment would improve access to cannabidiol and enhance the efficiency of the prescribing process.

5.4 The PBAC noted that there was a small population that benefits the use of cannabidiol for DS, that prescribers now have considerable experience with its use, and that the risk of prescribing to ineligible patients in this indication is low. Therefore, the PBAC considered a streamlined authority to be appropriate.

5.5 The PBAC recalled that, at its November 2024 meeting, it advised that the listings of fenfluramine, stiripentol and cannabidiol should be aligned, with no restrictions on combination use or patient age (fenfluramine Public Summary Document [PSD], November 2024).

5.6 The PBAC advised that changes to the cannabidiol listing should flow on to PBS listings for fenfluramine and stiripentol, for the treatment of DS (i.e. flow-on changes).

5.7 The PBAC advised that, given the small eligible population, the proposed changes were not expected to result in any additional cost to the PBS/RPBS.

5.8 The PBAC found that the criteria prescribed by the *National Health (Pharmaceuticals and Vaccines – Cost Recovery) Regulations 2022* for Pricing Pathway A were not met, as its recommendation to amend the clinical criteria for cannabidiol for the treatment of seizures associated with DS is not expected to address a high and urgent unmet clinical need.

5.9 The PBAC noted that this submission is not eligible for an Independent Review as it received a positive recommendation.

Outcome:
Recommended

6 Recommended listing

6.1 Amend existing listing as follows:

Additions are in *Italics* and deletions are in ~~strikethrough~~

MEDICINAL PRODUCT medicinal product pack	**PBS item code**	**Max. qty packs**	**Max. qty units**	**№.of Rpts**	**Available brands**
CANNABIDIOL					
cannabidiol 100 mg/mL oral liquid, 100 mL	12467E	1	1	5	Epidyolex
Restriction Summary 17572/ Treatment of Concept: 17521					
Concept ID (for internal Dept. use)	**Category / Program:** ☒ GENERAL - General Schedule (Code GE)				
	Prescriber type: ☒Medical Practitioners				
	Restriction type: ☒***Authority Required (Streamlined)*** ☒~~Authority Required (telephone/online PBS Authorities system)~~				
Prescribing rule level	~~**Administrative Advice:** *Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 888 333.*~~				
	Administrative Advice: Requests for increased quantities may be sought based on daily doses not exceeding 20 mg/kg/day (in line with the Product Information) for up to 4 weeks per dispensing.				
	Administrative Advice: No increase in the maximum number of repeats may be authorised.				
	Administrative Advice: Special Pricing Arrangements apply.				
	Indication: Severe myoclonic epilepsy in infancy (Dravet syndrome)				
	Clinical criteria:				
	Patient must have (as an initiating patient)/have had (as a continuing patient), generalised tonic-clonic seizures or generalised clonic seizures that are not adequately controlled with at least two other antiseizure medications				
	AND				
	Clinical criteria:				
	The treatment must be as adjunctive therapy to ~~*at least two*~~ other antiseizure medication~~s~~				
	Treatment criteria				
	Must be treated by a *prescriber who is either: (i) a* neurologist ~~**or**~~ *(ii) a paediatrician* if treatment is being initiated; or				
	Must be treated by a *prescriber who is either: (i) a* neurologist ~~**or**~~ *(ii) a paediatrician* if treatment is being continued or re-initiated; or				
	~~Must be treated by a paediatrician in consultation with a neurologist if treatment is being continued; or~~				
	Must be treated by a general practitioner in consultation with *either: (i)* a neurologist (ii) ~~**or**~~ *a* **paediatrician** if treatment is being continued				
	Prescribing Instructions*: Confirmation of eligibility for treatment with diagnostic reports must be documented in the patient's medical records.*				

Flow on changes:
To Fenfluramine for Severe myoclonic epilepsy in infancy (Dravet syndrome) – 14833Q updates in RED

MEDICINAL PRODUCT medicinal product pack	**PBS item code**	**Max. qty packs**	**Max. qty units**	**№.of Rpts**	**Available brands**
FENFLURAMINE					

fenfluramine hydrochloride 2.2 mg/mL oral liquid, 360 mL	14833Q	1	1	5	Fintepla

Restriction Summary 17523/ Treatment of Concept: 17457		
Concept ID (for internal Dept. use)		**Category / Program:** ☒ GENERAL - General Schedule (Code GE)
		Prescriber type: ☒Medical Practitioners
		Restriction type: ☒***Authority Required (Streamlined)*** ☒~~Authority Required (telephone/online PBS Authorities system)~~
		Administrative Advice: Cardiac monitoring must be carried out in accordance with the approved Product Information while on treatment with this drug for this condition.
Prescribing rule level		~~**Administrative Advice:** *Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 888 333.*~~
		Administrative Advice: No increase in the maximum quantity or number of units may be authorised.
		Administrative Advice: No increase in the maximum number of repeats may be authorised.
		Administrative Advice: Special Pricing Arrangements apply.
		Indication: Severe myoclonic epilepsy in infancy (Dravet syndrome)
		Clinical criteria:
		Patient must have (if initiating) generalised tonic-clonic seizures or generalised clonic seizures that are not adequately controlled with at least two other antiseizure medications; or
		Patient must have had (if continuing) generalised tonic-clonic seizures or generalised clonic seizures that are not adequately controlled with at least two other antiseizure medications
		AND
		Clinical criteria:
		The treatment must be as adjunctive therapy to ~~*at least two*~~ other antiseizure medication~~s~~
		Treatment criteria
		Must be treated by a *prescriber who is either: (i) a* neurologist ~~*or*~~ *(ii) a paediatrician* if treatment is being initiated; or
		Must be treated by a *prescriber who is either: (i) a* neurologist ~~*or*~~ *(ii) a paediatrician* if treatment is being continued or re-initiated; or
		~~Must be treated by a paediatrician in consultation with a neurologist if treatment is being continued; or~~
		Must be treated by a general practitioner in consultation with *either: (i)* a neurologist (ii) ~~*or*~~ *a paediatrician* if treatment is being continued
		Prescribing Instructions: *Confirmation of eligibility for treatment with diagnostic reports must be documented in the patient's medical records.*

Flow on changes:
To Stiripentol for Severe myoclonic epilepsy in infancy (Dravet syndrome) – updates in RED

MEDICINAL PRODUCT medicinal product pack	**PBS item code**	**Max. qty packs**	**Max. qty units**	**№.of Rpts**	**Available brands**
STIRIPENTOL					
stiripentol 250 mg capsule, 60	12103B	2	120	3	Diacomit®
stiripentol 500 mg capsule, 60	12107F	2	120	3	Diacomit®
stiripentol 250 mg powder for oral liquid, 60 sachets	12106E	2	120	3	Diacomit®
stiripentol 500 mg powder for oral liquid, 60 sachets	12088F	2	120	3	Diacomit®

Restriction Summary 1 17486/ Treatment of Concept: 17456	
Concept ID (for internal Dept. use)	**Category / Program:** ☒ GENERAL - General Schedule (Code GE)
	Prescriber type: ☒Medical Practitioners
	Restriction type: ☒Authority Required (Streamlined)
	Indication: Severe myoclonic epilepsy in infancy (Dravet syndrome)
	Clinical criteria:
	Patient must have (as an initiating patient)/have had (as a continuing patient), generalised tonic-clonic seizures or generalised clonic seizures that are not adequately controlled with at least two other antiseizure medications
	AND
	Clinical criteria:
	The treatment must be as adjunctive therapy to ~~*at least two*~~ other antiseizure medication~~s~~
	Treatment criteria
	Must be treated by a *prescriber who is either: (i) a* neurologist ~~*or*~~ *(ii) a paediatrician* if treatment is being initiated; or
	Must be treated by a *prescriber who is either: (i) a* neurologist ~~*or*~~ *(ii) a paediatrician* if treatment is being continued or re-initiated; or
	~~Must be treated by a paediatrician in consultation with a neurologist if treatment is being continued; or~~
	Must be treated by a general practitioner in consultation with *either: (i)* a neurologist (ii) ~~*or*~~ *a paediatrician* if treatment is being continued
	Prescribing Instructions: *Confirmation of eligibility for treatment with diagnostic reports must be documented in the patient's medical records.*

This restriction may be subject to further review. Should there be any changes made to the restriction the sponsor will be informed

7 Context for Decision

The PBAC helps decide whether and, if so, how medicines should be subsidised through the Pharmaceutical Benefits Scheme (PBS) in Australia. It considers applications regarding the listing of medicines on the PBS and provides advice about other matters relating to the operation of the PBS in this context. A PBAC decision in relation to PBS listings does not necessarily represent a final PBAC view about the merits of the medicine or the circumstances in which it should be made available through the PBS. The PBAC welcomes applications containing new information at any time.

8 Sponsor's Comment

The sponsor had no comment.