

An addendum to these minutes has been included at the end of the document.

7.08 TETRACOSACTIDE

Compound depot injection 1 mg in 1 mL Synacthen[®], Clinect Pty Ltd

1 Purpose of Application

- 1.1 The minor submission sought the derestriction of tetracosactide 1 mg/1 mL compound depot for the treatment of hypsarrhythmia and/or infantile spasms from a restricted benefit to an unrestricted benefit. The minor submission stated that this would ensure equity of access to patients requiring treatment, particularly those with multiple sclerosis (MS). This would expand the listing of tetracosactide to include the treatment of acute exacerbations in patients suffering from MS.
- 1.2 The minor submission also sought an approved ex-manufacturer price (AEMP) increase from \$68.40 to \$█.
- 1.3 The minor submission stated that, at the current PBS reimbursed price, supply of tetracosactide is unsustainable in the Australian market, and that the product will be delisted if its pricing is not revised.

2 Background

- 2.1 Tetracosactide is currently listed on the PBS as a restricted benefit listing for the treatment of hypsarrhythmia and/or infantile spasms.
- 2.2 Tetracosactide is an adrenocorticotrophic hormone (ACTH) replacement that was registered by the Therapeutic Goods Administration (TGA) on 2 August 1991 for the treatment of:

‘Neurological Diseases: Acute exacerbations in patients suffering from MS. Hypsarrhythmia, and or infantile spasms.
- 2.3 The minor submission states that, in the late stages of pricing negotiations with the local sponsor at the time, Mallinckrodt (the global manufacturer at the time) advised that it would supply worldwide markets at a significantly reduced price for the paediatric indication in order to establish a secure supply. Tetracosactide was therefore listed with an AEMP of \$80, rather than the originally requested AEMP of \$█, and this was accepted by the PBAC in December 2016.
- 2.4 Tetracosactide was first listed on the PBS as an unrestricted benefit. At its November 2017 meeting, the PBAC considered the sponsor’s request and recommended amending the unrestricted benefit to a restricted benefit for use in patients with hypsarrhythmia and/or infantile spasms, noting that this is a small patient group (tetracosactrin, November 2017 Public Summary Document (PSD)).

Public Summary Document – March 2021 PBAC Meeting with May 2021 Addendum

- 2.5 On 1 June 2018, tetracosactide received a 15 year anniversary price reduction with the introduction of the anniversary price reduction budget measure. The AEMP was reduced from \$80 to \$68.40.
- 2.6 In February 2019, the sponsor reported to the TGA a shortage due to manufacturing issues with an anticipated re-supply date of 1 February 2021¹. The pre-PBAC response stated that supply was expected to resume in Q3 2021.
- 2.7 Prior to the shortage, tetracosactide had an average of 5 PBS prescriptions per month (PBS data 1 January 2016 – 31 December 2018) and a total of 11 prescriptions and 8 prescriptions were dispensed in 2019 and 2020, respectively (PBS data 1 January 2019 – 31 December 2020). The pre-PBAC response stated that ‘the Director of Pharmacy at Westmead Children’s Hospital confirmed a continued role for tetracosactide in the management of infantile spasms. Over the period of shortage, access was sought to unregistered product via the Special Access Scheme, with different product strengths and formulation available over the period’.

¹ <https://apps.tga.gov.au/Prod/msi/Search/Details/tetracosactide-tetracosactrin>

Previous PBAC considerations

Committee-In-Confidence information

[REDACTED]	[REDACTED]	[REDACTED]	[REDACTED]
[REDACTED]	[REDACTED]	[REDACTED]	[REDACTED]
[REDACTED]	[REDACTED]	[REDACTED]	[REDACTED]
[REDACTED]	[REDACTED]	[REDACTED]	[REDACTED]
[REDACTED]	[REDACTED]	[REDACTED]	[REDACTED]

End Committee-In-Confidence information

For more detail on PBAC’s view, see section 6 PBAC outcome.

3 Requested listing

3.1 The minor submission requested the existing listing be amended from a restricted benefit to an unrestricted benefit.

For more detail on PBAC’s view, see section 6 PBAC outcome.

4 Comparator

4.1 The PBAC noted that the minor submission did not nominate any comparators, and claimed that the clinical algorithm for the requested indications would not be

impacted by the requested change in restriction level. The PBAC considered that there were no relevant comparators for this submission.

For more detail on PBAC's view, see section 6 PBAC outcome.

5 Consideration of the evidence

Sponsor hearing

5.1 There was no hearing for this item as it was a minor submission.

Consumer comments

5.2 The PBAC noted that no consumer comments were received for this item.

Clinical

5.3 The minor submission did not provide any clinical evidence to support the requested restriction change or price increase.

5.4 The PBAC noted the sponsor's pre-PBAC response that a couple of children's hospitals sought access to tetracosactide via the Special Access Scheme over the supply shortage period. The sponsor noted that supply has been limited and unreliable under the SAS and that the supply issues have subsequently impacted current treatment protocols. The sponsor further noted from one of the children's hospitals that there is still clinical relevance to the use of tetracosactide for the treatment of infantile spasms.

5.5 The PBAC recalled receiving advice from the Australian and New Zealand Child Neurology Society (ANZCNS), which advised that there was a clinical need in the paediatric population for tetracosactide in the treatment of hypsarrhythmia, and/or infantile spasm. The PBAC considered that more current advice is required from the ANZCNS.

Economic analysis

5.6 The submission used a cost plus analysis as the basis of the economic evaluation. The PBAC considered that this method is outdated and noted that it is not referenced in the current PBAC Guidelines.

5.7 The sponsor claimed that the cost of goods for an ampoule of Synacthen Depot is \$█. The requested AEMP is \$█ per ampoule.

5.8 As a minor submission, the economic analysis was not independently evaluated.

Financial estimates

5.9 The submission claimed that, due to changes in the treatment practice for MS over the past decade, the situation of patients who require treatment with tetracosactide in MS would occur in extremely rare situations where other treatments are not appropriate. The submission provided utilisation data which showed that PBS services for tetracosactide declined from < 500 scripts per year in 2010/2011 to < 500 scripts

per year in 2017/2018 following the addition of oral therapies and monoclonal antibodies to the PBS for the treatment of MS. The submission therefore estimated that the requested restriction change would have no net impact on the script numbers for tetracosactide.

5.10 The submission assumed that there would be no projected growth in script numbers given that the script volumes used in the estimates were from years when the medicine was unrestricted and not subject to shortages. The PBAC considered it was uncertain if this assumption is reliable.

5.11 The submission estimated that the net cost to the PBS/RPBS resulting from the requested restriction change and price increase would be 0 to < \$10 million per year, or 0 to < \$10 million over the first six years of listing.

Table 2: Estimated use and financial implications

	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
Estimated extent of use						
Number of scripts dispensed	█ ¹	█ ¹	█ ¹	█ ¹	█ ¹	█ ¹
Estimated financial implications for tetracosactide (requested price)						
Cost to PBS/RPBS	\$█ ²	\$█ ²	\$█ ²	\$█ ²	\$█ ²	\$█ ²
Copayments	\$█ ²	\$█ ²	\$█ ²	\$█ ²	\$█ ²	\$█ ²
Cost to PBS/RPBS less copayments	\$█ ²	\$█ ²	\$█ ²	\$█ ²	\$█ ²	\$█ ²
Estimated financial implications for tetracosactide (current listing)						
Cost to PBS/RPBS	-\$█ ²	-\$█ ²	-\$█ ²	-\$█ ²	-\$█ ²	-\$█ ²
Copayments	\$█ ²	\$█ ²	\$█ ²	\$█ ²	\$█ ²	\$█ ²
Cost to PBS/RPBS less copayments	-\$█ ²	-\$█ ²	-\$█ ²	-\$█ ²	-\$█ ²	-\$█ ²
Net financial implications						
Net cost to PBS/RPBS	\$█ ²	\$█ ²	\$█ ²	\$█ ²	\$█ ²	\$█ ²

The redacted values correspond to the following ranges:

¹ < 500

² \$0 to < \$10 million

5.12 As a minor submission, the financial estimates were not independently evaluated.

For more detail on PBAC's view, see section 6 PBAC outcome.

6 PBAC Outcome

6.1 The PBAC deferred the consideration of derestricting the listing of tetracosactide to an unrestricted benefit, pending further consultation with the ANZCNS.

6.2 The PBAC noted that the submission requested this restriction change to allow patients with MS to access PBS subsidised tetracosactide. The PBAC considered that the submission did not present sufficient evidence to support the clinical need in MS or the cost-effectiveness of tetracosactide as an unrestricted benefit. Furthermore, the PBAC considered that an unrestricted listing may lead to potential uses for tetracosactide outside of its TGA registered indication. The PBAC noted that the pre-

PBAC response stated that the sponsor was amenable to a future utilisation review to assess the impact of the proposed changes on projected volumes.

- 6.3 The PBAC considered that while there is some clinical need for tetracosactide on the PBS for MS, it has largely been replaced by prednisolone and methylprednisolone in therapy. The PBAC noted that there were other PBS listed treatments for MS and considered that there would be a low uptake of tetracosactide in the MS population.
- 6.4 The PBAC recalled the 2016 ANZCNS advice that there was a clinical need in the paediatric population for tetracosactide in the treatment of hypsarrhythmia, and/or infantile spasm. However, the PBAC noted that there has continued to be substantial supply issues and noted that tetracosactide has not been available on the PBS due to supply issues since February 2019 and would not be available until June 2021. The PBAC considered that the treatment practice may have changed as a result of the supply shortage and requested further advice to be sought from the ANZCNS to ascertain the current clinical use of tetracosactide. In particular, the ANZCNS advice would be sought to ascertain how patients may have managed during the shortage period and if the shortage has resulted in patients using treatment options other than tetracosactide.

Outcome:
Deferred

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.

Addendum to the March 2021 PBAC Minutes:

4.03 TETRACOSACTIDE

Compound depot injection 1 mg in 1 mL

Synacthen[®],

Clinect Pty Ltd

9 Background

- 9.1 At its March 2021 meeting, the PBAC deferred the consideration of derestricting the listing of tetracosactide to an unrestricted benefit, pending further consultation with the ANZCNS.
- 9.2 The ANZCNS provided advice on 6 May 2021 regarding the clinical place for tetracosactide on the PBS.

10 PBAC Outcome

- 10.1 The PBAC noted advice from the ANZCNS that while tetracosactide continues to be rarely used in the treatment of infantile spasms and Opsoclonus-Myoclonus Syndrome (OMS), there remains a clinical need for the treatment of refractory infantile spasms or for OMS that is unresponsive to other treatments. The PBAC advised that the delisting of tetracosactide would result in an unmet clinical need for patients with hypsarrhythmia and/or infantile spasms.
- 10.2 The PBAC noted that there is no clinical need for tetracosactide in the treatment of MS and therefore did not recommend any change to the current restriction for the treatment of hypsarrhythmia and/or infantile spasms.

Outcome:

Advice given

11 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.

12 Sponsor's Comment

The sponsor had no comment.