

5.14 AMINO ACID FORMULA WITH VITAMINS AND MINERALS WITHOUT VALINE, LEUCINE AND ISOLEUCINE, Sachets containing oral powder 28 g, 30, MSUD Lophlex®, Nutricia Australia Pty Ltd

1 Purpose of Application

- 1.1 The minor submission requested the Restricted Benefit listing of amino acid formula with vitamins and minerals without valine, leucine and isoleucine (MSUD Lophlex®) for the dietary management of Maple Syrup Urine Disease (MSUD).

2 Background

- 2.1 The Sponsor of MSUD Lophlex confirmed it meets the requirements for foods for medical purposes as set out under *The Australia New Zealand Food Standards Code – Standard 2.9.5: Food for Special Medical Purposes*.
- 2.2 MSUD Lophlex is a concentrated leucine, isoleucine and valine free protein substitute containing 20 g of protein equivalence (PE) per sachet for the dietary management of MSUD.
- 2.3 MSUD Lophlex has not previously been considered by the PBAC.

3 Requested listing

- 3.1 The submission did not propose a detailed restriction. The Secretariat proposed the following restriction based on the information provided in the submission.
- 3.2 Secretariat suggested additions are in italics and deletions are in strikethrough.

Name, Restriction, Manner of administration and form	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Proprietary Manufacturer	Name and
AMINO ACID FORMULA WITH VITAMINS AND MINERALS WITHOUT VALINE, LEUCINE AND ISOLEUCINE Sachets containing oral powder 28 g, 30	NEW	4	4	5	MSUD Lophlex®	Nutricia Australia Pty Ltd

Restriction Summary [new] / Treatment of Concept: [new]

Category / Program: GENERAL – General Schedule (Code GE)
Prescriber type: <input type="checkbox"/> Dental <input checked="" type="checkbox"/> Medical Practitioners <input checked="" type="checkbox"/> Nurse practitioners <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives
Restriction type/ Method:

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<input type="checkbox"/> Unrestricted benefit
<input checked="" type="checkbox"/> Restricted benefit
<input type="checkbox"/> Authority Required – In Writing
<input type="checkbox"/> Authority Required – Telephone/Electronic/Emergency
<input type="checkbox"/> Authority Required - Streamlined
Condition: Maple syrup urine disease
Indication: Maple syrup urine disease
Population criteria:
Patient must be aged 3 years or over

For more detail on PBAC’s view, see section 7 PBAC outcome.

4 Comparator

- 4.1 The minor submission nominated MSUD Express 20, MSUD Cooler 20, MSUD Lophlex LQ 20 and MSUD Maxamum as the main comparators.
- 4.2 The submission stated that although MSUD Cooler 20 is currently the most widely prescribed product for MSUD patients aged 3 years and above, MSUD Express 20 is expected to be the product that MSUD Lophlex is likely to replace due to both products being powders.

For more detail on PBAC’s view, see section 7 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.

Drug cost/patient/year: \$40,309.32

- 5.3 The above calculation of nutritional product cost per patient per year was derived from the DPMQ for one month’s supply (\$3,349.11¹) over 12 months, and assumed that a patient would receive the maximum quantity (4 packs) per dispensing, however this may not occur in practice due to variations in individual patient needs.
- 5.4 The submission derived a requested price based on a cost minimisation with the nominated comparators at the same price per gram of PE of \$1.33 at the approved ex-manufacturer price (AEMP).

¹ The DMPQ presented in the submission was incorrectly calculated to be \$3372.42

Estimated PBS usage & financial implications

- 5.5 The minor submission stated that the listing of MSUD Lophlex would not result in any new patients commencing dietary management for MSUD, and estimated there to be no financial implications of the listing.
- 5.6 The Sponsor estimated there would be a 20% reduction in the use of MSUD Maxamum, MSUD Lophlex LQ 20, MSUD Cooler 20 and MSUD Express 20 (or other MSUD equivalent supplements) if MSUD Lophlex is PBS listed.
- 5.7 The submission did not provide financial estimates as they claimed that the financial implications are impossible to calculate due to the small patient population.

For more detail on PBAC's view, see section 7 PBAC outcome.

6 NPWP Consideration

- 6.1 The NPWP accepted MSUD Express 20, MSUD Cooler 20, MSUD Lophlex LQ 20 and MSUD Maxamum as the comparators for MSUD Lophlex.
- 6.2 The NPWP considered that the requested age restriction was not required as prescribing is usually by specialists and nutritionists, and there was little risk of MSUD Lophlex being prescribed to younger patients.
- 6.3 The NPWP supported the listing of MSUD Lophlex as an additional treatment option for the dietary management of MSUD on a cost-minimisation basis with the nominated comparators.

For more detail on PBAC's view, see section 7 PBAC outcome.

7 PBAC Outcome

- 7.1 The PBAC recommended the Restricted Benefit listing of amino acid formula with vitamins and minerals without valine, leucine and isoleucine, MSUD Lophlex, for the management of MSUD on a cost-minimisation basis per gram of protein with the nominated comparators.
- 7.2 The PBAC noted that the NPWP accepted that MSUD Express 20, MSUD Cooler 20, MSUD Lophlex LQ 20 and MSUD Maxamum were relevant comparators.
- 7.3 The PBAC noted the NPWP view that the requested age restriction was not required, as prescribing is usually by specialists and nutritionists, and, due to the formulation, MSUD Lophlex is unlikely to be prescribed to younger patients. The PBAC agreed that the age restriction was not needed.
- 7.4 The PBAC noted that the requested comparators are currently listed on the PBS as Restricted Benefits, and recommended that MSUD Lophlex be listed on the PBS as a Restricted Benefit.
- 7.5 The PBAC recommended that under Section 101 (3BA) of the *National Health Act*

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1953, MSUD Lophlex should be treated as interchangeable on an individual patient basis with other amino acid formulas used for the treatment of MSUD.

- 7.6 The PBAC advised that MSUD Lophlex is suitable for prescribing by nurse practitioners.
- 7.7 The PBAC recommended that the Early Supply Rule should not apply.
- 7.8 The PBAC noted that its recommendation was on a cost-minimisation basis and advised that, because MSUD Lophlex is not expected address a high and urgent unmet clinical need given the presence of alternative therapies, the criteria prescribed by the *National Health (Pharmaceuticals and Vaccines – Cost Recovery) Regulations 2009* for Pricing Pathway A were not met.
- 7.9 The PBAC noted that this submission is not eligible for an Independent Review as it received a positive recommendation.

Outcome:

Recommended

8 Recommended listing

8.1 Add new item:

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Restriction type/ Method: <input checked="" type="checkbox"/> Restricted benefit
Condition: Maple syrup urine disease
Indication: Maple syrup urine disease

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

9 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.

10 Sponsor's Comment

The sponsor had no comment.