

## 5.12 AMINO ACID FORMULA WITH VITAMINS AND MINERALS WITHOUT METHIONINE, Sachets containing oral powder 28 g, 30, HCU 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 methionine (HCU Lophlex®) for the dietary management of pyridoxine non-responsive homocystinuria (HCU).

### 2 Background

- 2.1 The Sponsor of HCU 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 HCU Lophlex is a methionine free protein substitute containing 20 g of protein equivalence (PE) per sachet for the dietary management of HCU.
- 2.3 HCU 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	No. of Rpts	Proprietary Manufacturer	Name and
AMINO ACID FORMULA WITH VITAMINS AND MINERALS WITHOUT METHIONINE Sachets containing oral powder 28 g, 30	NEW	4	4	5	HCU Lophlex®	Nutricia Australia Pty Ltd

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

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

<b>Indication:</b> Pyridoxine non-responsive homocystinuria
<b>Population criteria:</b>
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 HCU Express 15, HCU Cooler 20, HCU Lophlex LQ 20 and XMET Maxamum as the main comparators.
- 4.2 The submission stated that although HCU Cooler 20 is currently the most widely prescribed product for HCU patients aged 3 years and above, HCU Express 15 is expected to be the product that HCU Lophlex is likely to replace due to both products being powders.

*For more detail on PBC'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: \$40,189.32***

- 5.3 The above calculation of nutritional product cost per patient per year was derived from the DPMQ for one month's supply (\$3,349.11<sup>1</sup>) 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).

### ***Estimated PBS usage & financial implications***

- 5.5 The minor submission stated that the listing of HCU Lophlex would not result in any new patients commencing dietary management for HCU, and estimated there to be no financial implications of the listing.

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<sup>1</sup> The DPMQ presented in the submission was incorrectly calculated to be \$3372.42

- 5.6 The Sponsor estimated there would be a 20% reduction in the use of HCU Lophlex LQ 20, HCU Cooler 20 and HCU Express 15 (or other HCU equivalent supplements) if HCU 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 HCU Express 15, HCU Cooler 20, HCU Lophlex LQ 20 and XMET Maxamum as the comparators for HCU Lophlex.
- 6.2 The NPWP noted that HCU Lophlex has a higher content of key nutrients than all comparators except for XMET Maxamum, and is most likely to be used for teenagers and adults.
- 6.3 The NPWP considered that the requested age restriction was not required as prescribing is usually by specialists and nutritionists, and, due to the strength of the formulation, HCU Lophlex is most likely to be used in teenagers and adults.
- 6.4 The NPWP supported the listing of HCU Lophlex as an additional treatment option for the dietary management of pyridoxine non-responsive HCU on a cost-minimisation basis with the nominated comparators (para 6.1).

*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 methionine, HCU Lophlex, for the management of pyridoxine non-responsive homocystinuria (HCU) on a cost-minimisation basis per gram of protein with the nominated comparators.
- 7.2 The PBAC noted that the NPWP accepted that HCU Express 15, HCU Cooler 20, HCU Lophlex LQ 20 and XMET 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, HCU Lophlex will likely be used in teenagers and adults. 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 HCU 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 1953*, HCU Lophlex should be treated as interchangeable on an individual patient basis with other amino acid formulas used for the treatment of HCU.

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- 7.6 The PBAC advised that HCU 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 HCU 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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<b>Restriction type/ Method:</b> <input checked="" type="checkbox"/> Restricted benefit
<b>Condition:</b> Pyridoxine non-responsive homocystinuria
<b>Indication:</b> Pyridoxine non-responsive homocystinuria

***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.