

## 5.15 AMINO ACID FORMULA WITH FAT, CARBOHYDRATE WITHOUT METHIONINE

Tablets, 0.91 g, 462

HCU Easy Tablet<sup>®</sup>, Orpharma Pty Ltd

### 1 Purpose of Application

- 1.1 The minor submission requested a Restricted Benefit listing for a new form of amino acid formula with fat, carbohydrate without methionine (HCU Easy<sup>®</sup>) for the dietary management of pyridoxine non-responsive homocystinuria (HCU).

### 2 Requested listing

- 2.1 The submission requested the following new listing:
- 2.2 Suggestions and additions proposed by the Secretariat to the requested listing are added in italics and suggested deletions are crossed out with strikethrough.

Name, Restriction, Manner of administration and form	Max. Qty	No. of Rpts	Dispensed Price for Max. Qty	Proprietary Name and Manufacturer
AMINO ACID FORMULA WITH FAT, CARBOHYDRATE WITHOUT METHIONINE Tablet: modified release, 6 x 77 g	5	5	\$2,952.29*	HCU Easy Tablet <sup>®</sup> <del>QH</del> Orpharma Pty Ltd

Tablet: modified release, 6 x 77 g

\* The DPMQ proposed in the minor submission for MSUD Easy Tablet (\$3,609.89) is derived from the AEMP for MSUD Express 20 for the dietary management of MSUD prior to the application of any relevant Statutory Price Reductions. In the overview, the relevant F1 Statutory Price Reductions have been applied to the requested listing to enable a relevant comparison to be made with MSUD Express 20 (see paragraphs 5.4-6).

<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>PBS Indication:</b>	Pyridoxine non-responsive homocystinuria
<b>Restriction Level / Method:</b>	<input checked="" type="checkbox"/> Restricted benefit <input type="checkbox"/> Authority Required – In Writing <input type="checkbox"/> Authority Required – Telephone/Electronic/Emergency <input type="checkbox"/> Streamlined
<b>Administration Advice:</b>	<i>This product does not contain any vitamins, minerals or trace elements.</i>

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

### 3 Background

- 3.1 The sponsor of HCU Easy 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*.

- 3.2 HCU Easy is a methionine-free protein substitute containing 0.91 g of protein equivalence (PE) per tablet for the treatment of pyridoxine non-responsive HCU.
- 3.3 The submission claimed that HCU Easy provides a convenient and palatable treatment option that will enhance adherence to therapy and is likely to provide benefits due to improved HCU control (over the comparators).

## 4 Comparator

- 4.1 The minor submission nominated HCU Cooler 20 as the main comparator, stating that it is the most commonly used amino acid formula without methionine product for treating HCU, and is therefore the most likely product to be replaced.
- 4.2 There are no similar tablet formulations currently PBS-listed. All current PBS-listed amino acid formulations for HCU are either oral liquids or powders for oral liquids.
- 4.3 The submission stated the PBS-listed products HCU Cooler 10 or 15, and HCU Lophlex LQ, are also indicated for the dietary management of pyridoxine non-responsive HCU. However, since all products are priced the same per protein equivalence, there are no cost implications for market displacement of either of these products by HCU Easy.
- 4.4 HCU Easy does not include any vitamins, minerals or trace elements, however the submission claimed HCU Easy is a complete substitute for any currently PBS-listed nutritional product indicated for HCU.

*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: \$35,427.48***

- 5.3 The submission determined the proposed DPMQ based on a cost minimisation analysis versus HCU Cooler 20, based on protein equivalent (PE) content (see Table 1).
- 5.4 The estimated nutritional product cost/patient/year would be \$35,427.48, based on a DPMQ of \$2,952.29 and 12 scripts per year.

**Table 1: Calculation of AEMP and DPMQ of HCU Easy based on cost equivalence to the comparator**

Product	PBS Pack	PE per PBS pack (g)	PE per Max Qty (g)	AEMP per pack <sup>a</sup>	PBS Max Qty	DPMQ <sup>a</sup>
HCU Easy tablets	77 tablet pack x 6 packs	420.4	2,102	\$560.03	5	\$2,952.29

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Product	PBS Pack	PE per PBS pack (g)	PE per Max Qty (g)	AEMP per pack <sup>a</sup>	PBS Max Qty	DPMQ <sup>a</sup>
HCU Cooler 20	Oral Liquid 174mL, 30 cans	600	2,400	\$799.25	4	\$3,349.15

<sup>a</sup> Cost-minimised to price of HCU Cooler 20 on 1 August 2019

Source: HCU Easy – Attachment 4 – financial estimates Microsoft Excel spreadsheet.

- 5.5 The submission sought HCU Easy to be considered under the pricing arrangements agreed under Clause 5.7 of the Strategic Agreement between Medicines Australia and the Commonwealth of Australia (the Strategic Agreement). The submission requested that HCU Easy not be subject to any statutory price reductions that have previously been applied to HCU Cooler 20.
- 5.6 The application of Clause 5.7 of the Strategic Agreement is a matter for the Minister (or delegate) and is not a matter for the PBAC. In Table 2 the relevant F1 Statutory Price Reductions have been applied to the requested listing to enable a relevant comparison to be made with HCU Cooler 20.
- 5.7 The DPMQ proposed in the submission for HCU Easy (\$3,599.59) is derived from the AEMP for HCU Cooler 20 for pyridoxine non-responsive HCU prior to the application of any relevant Statutory Price Reductions.

**Estimated PBS usage & financial implications**

- 5.8 The minor submission stated that usage of HCU Easy would be in place of other PBS-listed formulas for HCU and, thus, there is ‘no significant expected net financial impact of listing’. The submission assumed the current projected annual growth for the comparator to be 20% in 2020 and 2021, followed by 10% in 2022 and 5% thereafter. It also assumed HCU Easy would displace the comparator at the rate of 5% in 2020 and 2021, then 10% per year thereafter. The submission stated that 1.14 scripts of HCU Easy tablet were equivalent to 1 script of HCU Cooler 20, based on the different PE content in the PBS maximum quantity for each product.
- 5.9 Based on the estimates of utilisation in the minor submission and a DPMQ of \$2.952.29, listing of HCU Easy tablet is expected to be cost-neutral to the PBS.

**Table 2: Estimated use and financial implications**

	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
<b>Estimated extent of use</b>						
Number of scripts dispensed <sup>a</sup>	█	█	█	█	█	█
<b>Estimated financial implications of HCU Easy tablets</b>						
Cost to PBS/RPBS	\$ █	\$ █	\$ █	\$ █	\$ █	\$ █
Copayments	-\$ █	-\$ █	-\$ █	-\$ █	-\$ █	-\$ █
Cost to PBS/RPBS less copayments	\$ █	\$ █	\$ █	\$ █	\$ █	\$ █
<b>Estimated financial implications for HCU Cooler 20</b>						
Cost to PBS/RPBS	-\$ █	-\$ █	-\$ █	-\$ █	-\$ █	-\$ █
Copayments	\$ █	\$ █	\$ █	\$ █	\$ █	\$ █

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	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
Cost to PBS/RPBS less copayments	-\$██████	-\$██████	-\$██████	-\$██████	-\$██████	-\$██████
<b>Net financial implications<sup>b</sup></b>						
<b>Net cost to PBS/RPBS</b>	<b>\$██████</b>	<b>\$██████</b>	<b>\$██████</b>	<b>\$██████</b>	<b>\$██████</b>	<b>\$██████</b>

<sup>a</sup> Scripts per year as estimated by the submission, adjusted by the equivalent PE amount to the comparator.

<sup>b</sup> The relevant F1 Statutory Price Reductions have been applied to the price of the requested listing to enable a relevant comparison to be made with HCU Cooler 20 (see paragraphs 5.4-6). The submission estimated that if HCU Easy is listed at the DPMQ proposed in the submission (of \$3,599.59), the net cost to the PBS and RPBS over the first 6 years of listing would be \$██████.

Source: HCU Easy – Attachment 4 – financial estimates Microsoft Excel spreadsheet

The redacted table shows that at Year 6, the estimated number of scripts dispensed was less than 10,000 and the net cost to the PBS would be less than \$10 million.

5.10 As a minor submission, the financial estimates have not been independently evaluated.

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

## 6 NPWP Consideration (and sponsor’s further clarification)

6.1 The Nutritional Products Working Party (NPWP) accepted HCU Cooler 20 as the comparator for HCU Easy.

6.2 The NPWP noted HCU Easy does not contain any vitamins, minerals or trace elements and therefore patients would have to achieve their recommended daily intake (RDI) of these by other means, such as through diet or supplements. Some of these vitamin, mineral and trace element supplements, such as Phlexy-Vits or Paediatric Seravit (and other alternatives), are subsidised on the PBS. Therefore, the NPWP considered that while the requested cost-minimisation may be an appropriate basis for listing, this did not necessarily mean a listing would be cost neutral to either patients or the PBS as some patients would require PBS-subsidised supplements.

6.3 The NPWP considered the use of the tablet formulation was likely to be low because of the large number of tablets patients had to take and the need to obtain vitamins and minerals from other sources.

6.4 The NPWP supported the listing of HCU Easy as an additional treatment option for the dietary management of pyridoxine non-responsive HCU, on a cost-minimisation basis with HCU Cooler 20 per gram of protein equivalent.

6.5 The NPWP supported adding a note to the listing stating, ‘This product does not contain any vitamins, minerals or trace elements.

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

## 7 PBAC Outcome

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- 7.1 The PBAC recommended the Restricted Benefit listing of amino acid formula with fat, carbohydrate without methionine, HCU Easy, for the dietary management of pyridoxine non-responsive homocystinuria (HCU).
- 7.2 The PBAC noted the NPWP supported the proposed listing as a treatment option for the dietary management of pyridoxine non-responsive HCU. The PBAC also noted the NPWP advice that HCU Easy does not contain any vitamins, minerals or trace elements, therefore patients would have to meet their dietary needs through other sources, which may include use of PBS-subsidised vitamin, mineral and trace element supplements such as Phlexy-vits and Paediatric Seravit.
- 7.3 The PBAC noted the NPWP supported an administrative note being added to the restriction advising prescribers of the lack of vitamins, minerals and trace elements, to note the difference from all currently listed PBS products for the treatment of HCU.
- 7.4 The PBAC accepted that HCU Cooler 20 was a relevant comparator for HCU Easy. However, the PBAC also considered other amino acid formulas (such as HCU Cooler 10 or 15, and HCU Lophlex LQ) also used for the treatment of pyridoxine non-responsive HCU were also relevant comparators.
- 7.5 Given the likely low use of this products, the PBAC considered that while there may be an additional per-patient cost to the PBS if additional vitamin, mineral and trace element supplements are prescribed, any additional overall cost to the PBS was likely to be low. On balance, the PBAC considered that HCU Easy was a suitable alternative amino acid formula for some patients and would be acceptably cost-effective if it costs no more per mg of protein when compared to the lowest priced alternative amino acid formula for HCU.
- 7.6 The PBAC recommended that, under Section 101(3BA) of the *National Health Act 1953*, HCU Easy should be treated as interchangeable on an individual patient basis with other amino acid formulas used for the treatment of HCU.
- 7.7 The PBAC advised that HCU Easy is suitable for prescribing by nurse practitioners, as is the case with other nutritional products.
- 7.8 The PBAC recommended that the Early Supply Rule should not apply since it has generally been the PBAC's view that nutritional product listings be exempt.
- 7.9 The PBAC noted that this submission is not eligible for Pricing Pathway A under *National Health (Pharmaceutical and Vaccines – Cost Recovery) Regulations 2009* because HCU Easy is not expected to provide a substantial and clinically relevant improvement in efficacy, or reduction of toxicity, over any alternative therapy and is not expected to address a high and urgent unmet clinical need.
- 7.10 The PBAC noted that this submission is not eligible for an Independent Review because it received a positive recommendation.

**Outcome:**

Recommended

## 8 Recommended listing

### 8.1 Add new item:

Name, Restriction, Manner of administration and form	Max. Qty	No. of Rpts	Proprietary Name and Manufacturer	
AMINO ACID FORMULA WITH FAT, CARBOHYDRATE WITHOUT METHIONINE Tablet: modified release, 6 x 77g	5	5	HCU Easy®	Orpharma Pty Ltd

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<b>Restriction Level / Method:</b>	<input checked="" type="checkbox"/> Restricted benefit <input type="checkbox"/> Authority Required – In Writing <input type="checkbox"/> Authority Required – Telephone/Electronic/Emergency <input type="checkbox"/> Streamlined
<b>Administration Advice:</b>	This product does not contain any vitamins, minerals or trace elements.

*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.